Guide to Expanded Access

Johnson&Johnson

About this guide

For patients who are seriously or terminally ill who are not eligible for a clinical trial and whose disease has exhausted available treatment options, Expanded Access (EA) may be a potential option to explore with their physician. In response to patient feedback, Johnson & Johnson first created this Guide to Expanded Access (EA Guide) in 2021 to support patients, caregivers and healthcare providers seeking information about Expanded Access. In collaboration with patient representatives, this Guide was created to support anyone in search of country-specific information about Expanded Access, also known as Compassionate Use, or Pre-Approval Access (PAA).

This second edition of the guide now includes country-specific information for countries in Asia, Europe, Nordics, the United Kingdom, as well as Canada and the United States. It includes people friendly information such as whether Expanded Access is an option, as well as the process for requests in each country.

How to use this guide

Information for each country may be accessed from the Table of Contents by clicking on links to each section.

A glossary of key terms is also included. If you are unfamiliar with Expanded Access, you may find it helpful to review the glossary first. To go back and forth between the Table of Contents and the Glossary, click the icons at the bottom of each page.

Additional resources about Expanded Access

For more information about Expanded Access at Johnson & Johnson as well as other tools and resources, please visit https://www.janssen.com/compassionate-use-pre-approval-access.

About this guide (cont.)

Question, comment?
Patient and caregiver feedback will continue to inform the content in this Guide. We sincerely hope you will share your thoughts and experience with the EA Guide and encourage you to send us an email at PAASupport@its.jnj.com to share your thoughts.

Acknowledgement & appreciation
Johnson & Johnson Patient Strategies & Solutions would like to thank our colleagues at Bionical Emas for their strong collaboration and shared patient focus in bringing this second edition Guide to patients, caregivers and healthcare providers.

Disclaimers
The information in this Guide is intended solely for educational and informational purposes. It is not intended as medical or healthcare advice or to be used for medical diagnosis or treatment for any individual problem. It is also not intended as a substitute for professional advice and services from a qualified healthcare provider familiar with your unique facts. Always seek the advice of your doctor or other qualified healthcare provider regarding any medical condition and before starting any new treatment.

We assume no responsibility for any consequence relating directly or indirectly to any action or inaction you take based on the information, or other material provided as part of the Services. While we strive to keep the information provided by the Services to be accurate, complete, and up to date, we do not give any assurances, and will not be responsible for, any damage or loss related to the accuracy, completeness, or timeliness of the information provided as part of the Services.

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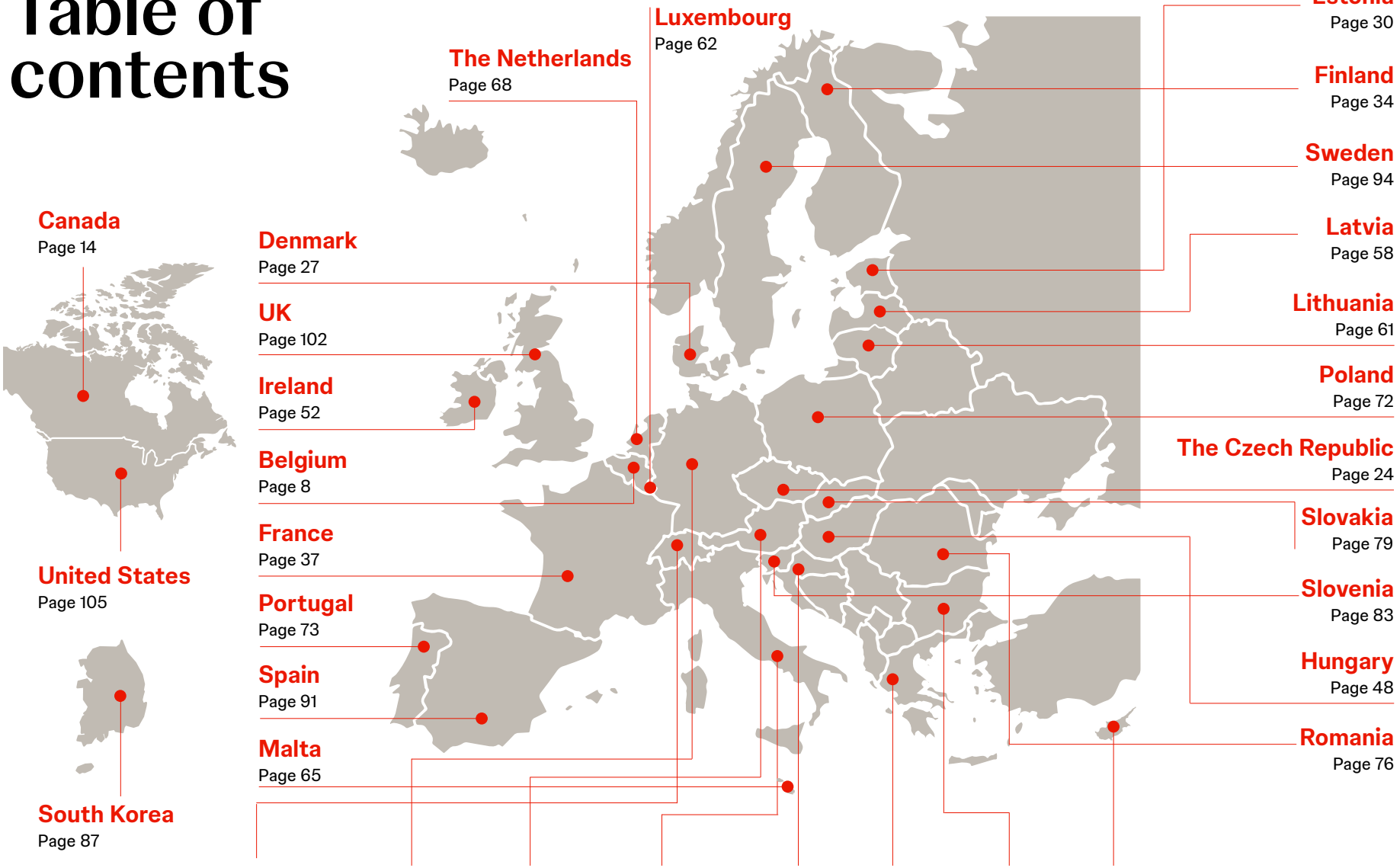

Patient guide

Austria

1. Is Expanded Access a potential option in Austria?

Yes, it is possible for an investigational medicine that is not approved for use in Austria to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Austria?

In Austria, to be considered for Expanded Access, a patient must have a serious or life-threatening illness and not able to be treated with available medicines.

Expanded Access may be provided for an individual patient, called “Named Patient” or for a group of patients, called a “Compassionate Use Program”. For compassionate use programs, patients must also be unable to join a clinical trial.

The treating physician must have approval from the pharmaceutical company to use the investigational medicine for their patient but does not need to gain approval from the Austrian Federal Office for Safety in Health Care (BASG), the medicines regulatory agency in Austria.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Austria (cont.)

4. What is the process for requesting Expanded Access in Austria?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the company approves the request, the treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Austria for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Austrian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Austria (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Austria the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Austria?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Austria?

Further information regarding the Expanded Access pathways in Austria can be found on the BASG website. Please note that the information on the website is intended for healthcare professionals. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Belgium

1. Is Expanded Access a potential option in Belgium?

Yes, it is possible for an investigational medicine that is not approved for use in Belgium to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Belgium?

For Expanded Access in Belgium, the investigational medicine must be provided within a program known as a 'compassionate use program'. A compassionate use program is set up voluntarily by the pharmaceutical company providing the investigational medicine. The program requires approval from the Federal Agency for Medicines and Health Products (FAMHP), the medicines regulatory agency in Belgium.

For a compassionate use program to be set up, the investigational medicine must:

- be intended for a group of patients with a serious or life-threatening condition and who cannot be treated with available approved for use medicines and are not eligible to participate in a clinical trial
- currently studied in clinical trials or is being reviewed for approval
- have sufficient evidence from clinical trials

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Belgium (cont.)

4. What is the process for requesting Expanded Access in Belgium?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the request is approved, the treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is open in Belgium for the investigational medicine in the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Belgian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Belgium (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Belgium, the medicine may potentially be made available through Expanded Access for a different condition. The requirements and processes are similar to those of a compassionate use program.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Belgium?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Belgium?

Further information regarding the Expanded Access pathways in Belgium can be found on the FAMHP website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Bulgaria

1. Is Expanded Access a potential option in Bulgaria?

Yes, it is possible for an investigational medicine that is not approved for use in Bulgaria to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Bulgaria?

For Expanded Access in Bulgaria, the investigational medicine must be provided within a program called a 'compassionate use program'. A compassionate use program is set up voluntarily by the pharmaceutical company providing the investigational medicine. The program requires approval from the Bulgarian Drug Agency (BDA), the medicines regulatory agency in Bulgaria, before patients can be treated.

For a compassionate use program, the investigational medicine must:

- be intended for a group of patients with a serious or life-threatening condition who cannot be treated with available approved for use medicines and are not eligible to participate in a clinical trial
- have entered the application process to gain approval or has sufficient evidence of potential efficacy and safety from clinical trials

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Bulgaria (cont.)

4. What is the process for requesting Expanded Access in Bulgaria?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the request is approved, the treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is open in Bulgaria for the investigational medicine in the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

In a compassionate use program in Bulgaria, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Bulgarian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Bulgaria (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Bulgaria, the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Bulgaria?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Bulgaria?

Further information regarding the Expanded Access pathways in Bulgaria can be found on the BDA website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Canada

1. Is Expanded Access a potential option in Canada?

Yes, it is possible for an investigational medicine that is not approved for use in Canada to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Canada?

Health Canada, the medicines regulatory agency in Canada, may authorize the use of an investigational medicine following a request from the patient's treating physician, under a program known as the 'Special Access Program'.

The investigational medicine must be intended for an individual who:

- has a serious or life-threatening condition and cannot be treated with medicines approved for use in Canada and
- is unable to participate in a clinical trial.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Canada (cont.)

4. What is the process for requesting Expanded Access in Canada?

In all cases, the treating physician must submit a request for the investigational medicine to Health Canada and the pharmaceutical company. If the request is approved, Health Canada issues an authorization letter to the company and physician.

If the company agrees to supply the investigational medicine, the treating physician must inform the patient about its potential risks and potential benefits. Additionally, the patient, or their legal representative, will usually sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Canada for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Canadian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Canada (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Canada, the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Canada?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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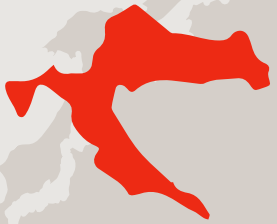
9. Where can I read more about Expanded Access in Canada?

Further information regarding the Special Access Program in Canada can be found on the Health Canada website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Croatia



1. Is Expanded Access a potential option in Croatia?

Yes, it is possible for an investigational medicine that is not approved for use in Croatia to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Croatia?

In Croatia, to be considered for Expanded Access, a patient must have a serious or life-threatening condition, not able to be treated with available medicines, and ineligible for a clinical trial.

The healthcare institution where the patient is treated will require a signed letter from the pharmaceutical company, confirming the company will provide the investigational medicine for the patient's treatment.

The healthcare institution must gain approval from the Ministry of Health (the health authority in Croatia) and from the Ethics Committee for the use of the investigational medicine for their patient.

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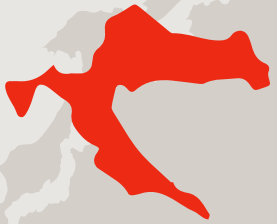
3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Croatia (cont.)



4. What is the process for requesting Expanded Access in Croatia?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the company approves the request, a signed letter is provided to the healthcare institution confirming the investigational medicine will be provided for treating the patient.

The healthcare institution must also gain approval from the Ethics Committee and then submit an application to the Ministry of Health in Croatia.

If the request is approved by the Ministry of Health, the treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Croatia for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

Croatia (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

The investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Croatian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Croatia, the medicine may be made available through Expanded Access for a different condition, if certain requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in Croatia?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Croatia (cont.)

9. Where can I read more about Expanded Access in Croatia?

Further information regarding the Expanded Access pathway in Croatia can be found on the Ministry of Health website. Please note that the information on the website is intended for healthcare professionals. Any questions patients, families and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Cyprus

1. Is Expanded Access a potential option in Cyprus?

Yes, it is possible for an investigational medicine that is not approved for use in Cyprus to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Cyprus?

The Ministry of Health - Pharmaceutical Services, the medicines regulatory agency in Cyprus, may authorize the use of an investigational medicine for an individual patient, following a request from the patient's treating physician.

The investigational medicine must be required to meet the unmet medical needs of the individual patient and be used under the treating physician's direct responsibility.

In addition to approval from the Ministry of Health, the treating physician must have approval from the pharmaceutical company to use the investigational medicine for their patient.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Cyprus (cont.)

4. What is the process for requesting Expanded Access in Cyprus?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the request is approved, the treating physician must gain approval from the Ministry of Health to import the investigational medicine.

The treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will usually be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Cyprus for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

The Pharmaceutical Council determines how long investigational medicine is provided free of charge from the pharmaceutical company developing and manufacturing it, which is typically for the duration of the treatment.

Cyprus (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Cyprus, the medicine cannot usually be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Cyprus?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Cyprus?

Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Czech Republic (Czechia)

1. Is Expanded Access a potential option in Czechia?

Yes, it is possible for an investigational medicine that is not approved for use in Czechia to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Czechia?

For Expanded Access in Czechia, the investigational medicine must be provided within a program called a 'Specific Therapeutic Program' or 'STP'.

An STP is set up voluntarily by the pharmaceutical company providing the investigational medicine and must be approved by the central health authority (Ministry of Health) and the medicines regulatory agency (State Institute for Drug Control [SUKL]) in Czechia. An STP can only be set up if the investigational medicine is under review for approval or being studied in clinical trials for the condition the medicine is being requested.

In an STP, the investigational medicine must be intended for a group of patients:

- with a life-threatening condition or a severe or chronically debilitating condition and,
- who cannot be treated with available approved for use medicines and,
- who are not able to join a clinical trial.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Czech Republic (cont.)

4. What is the process for requesting Expanded Access in Czechia?

To enter an STP, the treating physician needs to confirm the patient meets the eligibility criteria for the program as well as request the investigational medicine from the pharmaceutical company.

If a request is approved, the treating physician will inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if an STP is open in Czechia for the investigational medicine in the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Czechia healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Czech Republic (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Czechia, the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Czechia?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Czechia?

Further information regarding Specific Therapeutic Programs in Czechia can be found on the SUKL website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Denmark

1. Is Expanded Access a potential option in Denmark?

Yes, it is possible for an investigational medicine that is not approved for use in Denmark to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Denmark?

In special circumstances, a patient can be considered for Expanded Access based on medical need and if the patient cannot be treated with medicines that have been approved for use and available in Denmark.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

4. What is the process for requesting Expanded Access in Denmark?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company. If the request is approved, the treating physician must obtain an authorization known as a 'compassionate use permit' from the Danish Medicines Agency (DKMA), the medicines regulatory agency in Denmark.

The treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

Denmark (cont.)

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Denmark for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Danish healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Denmark the medicine cannot usually be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with the treating physician.

Denmark (cont.)

8. What are the key considerations for requesting Expanded Access when the patient does not live in Denmark?

Due to a variety of circumstances, sometimes patients seek to gain, or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in Denmark?

Further information regarding the compassionate use permit pathway in Denmark can be found on the DKMA website. Please note that the information on the website is intended for healthcare professionals. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Estonia

1. Is Expanded Access a potential option in Estonia?

Yes, it is possible for an investigational medicine that is not approved for use in Estonia to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Estonia?

Expanded Access in Estonia may be provided within a program called a 'compassionate use program'. A compassionate use program is set up voluntarily by the pharmaceutical company developing the investigational medicine. The program requires approval from the State Agency of Medicines (SAM), the medicines regulatory agency in Estonia, before patients can start treatment.

For a compassionate use program, the investigational medicine must:

- be intended for patients with a life-threatening or serious condition who cannot be treated with medicines that are approved for use, and who are not eligible to participate in a clinical trial
- be under review for approval or being studied in clinical trials
- have sufficient evidence of potential efficacy and safety.

The treating physician must gain approval from the pharmaceutical company and the SAM to use the investigational medicine for their patient within a compassionate use program.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Estonia (cont.)

4. What is the process for requesting Expanded Access in Estonia?

The treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If a compassionate use program is in place and the company approves the request, the treating physician applies to the SAM for a permit to use the investigational medicine. SAM decides whether to allow the use of the investigational medicine for the patient within 14 days, but it can take up to 30 days.

The treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is open in Estonia for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

Estonia (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

In a compassionate use program in Estonia the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary it is typically provided until the medicine is approved for use and available to be prescribed by a treating physician within the Estonian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Estonia, the medicine can be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in Estonia?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Estonia (cont.)

9. Where can I read more about Expanded Access in Estonia?

Further information regarding the compassionate use pathway in Estonia can be found on the SAM website. Please note that the information on this website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Finland

1. Is Expanded Access a potential option in Finland?

Yes, it is possible for an investigational medicine that is not approved for use in Finland to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Finland?

Expanded Access is possible in Finland for exceptional medical reasons, where no other treatment is appropriate or other available treatments are not effective, under the Special Permit process.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a healthcare professional responsible for the patient's care is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

4. What is the process for requesting Expanded Access in Finland?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the company approves the use of the investigational medicine for the patient, the hospital submits a Special Permit application to Fimea, which generally processes the request within 30 days of receipt, or as quickly as possible in urgent cases.

Finland (cont.)

If permission is granted, the treating physician provides the patient with information on the safe and correct use of the investigational medicine and informs the patient about potential risks and potential benefits. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Finland for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Finnish healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Finland (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Finland the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Finland?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Finalnd?

Further information regarding the Special Permit process in Finland can be found on the Fimea website. Please note that the information on the website is intended for healthcare professionals. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

France

1. Is Expanded Access a potential option in France?

Yes, it is possible for an investigational medicine that is not approved for use in France to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in France?

In France, Expanded Access can be provided in special cases for patients with a serious or rare condition when there is an urgent unmet medical need. There must be sufficient evidence of the potential efficacy and safety of the investigational medicine for the patient's condition.

An 'early access authorization' must be granted by the National Authority of Health (HAS), an independent public body in France, following a request from the pharmaceutical company developing the investigational medicine.

For an early access authorization, the investigational medicine must be expected to be innovative. For example, it may be the first available medicine to treat the condition.

Once an early access authorization has been granted, all patients who meet the defined criteria for the early access can be treated without further approval.

If there is no early access authorization in place and the investigational medicine is early in its development, the French Agency for the Safety of Medicines and Health Products (ANSM), the medicines regulatory agency in France, may grant a 'compassionate access authorization' following a request from the patient's treating physician. A compassionate access authorization can be granted for an individual patient with an urgent unmet medical need if the company developing the investigational medicine also agrees to its supply for the patient and commits to apply for an early access authorization within 1 year.

France (cont.)

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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4. What is the process for requesting Expanded Access in France?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If an early access authorization is in place and the patient meets the eligibility criteria, the patient can begin treatment.

If an early access authorization is not in place, the physician must apply to ANSM for a compassionate access authorization for an individual patient. Treatment can only be initiated once the compassionate use authorization is granted.

The treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

France (cont.)

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if an early access authorization has been granted for the investigational medicine for the relevant condition in France
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

An investigational medicine is often provided free of charge by the pharmaceutical company developing and manufacturing it. If it is not provided free of charge, an investigational medicine with an early access or compassionate access authorization is automatically fully covered by health insurance until the medicine is reimbursed in France. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in France, the medicine can only be made available through Expanded Access for a different condition if an early access authorization is in place for that condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

France (cont.)

8. What are the key considerations for requesting Expanded Access when the patient does not live in France?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in France?

Further information regarding the Expanded Access pathways in France can be found on the HAS website and ANSM website. Please note that some of the information is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Germany

1. Is Expanded Access a potential option in Germany?

Yes, it is possible for an investigational medicine that is not approved for use in Germany to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Germany?

For Expanded Access in Germany, the investigational medicine must be provided within a program called a 'compassionate use program'. A compassionate use program is set up voluntarily by the pharmaceutical company providing the investigational medicine and must be approved by the relevant regulatory agency in Germany. In Germany, there are two medicines regulatory agencies, the Federal Institute for Drugs and Medical Devices (BfArM) and the Paul-Ehrlich Institute (PEI), each responsible for different types of medicines.

For a compassionate use program, the investigational medicine must:

- be intended for patients with a life-threatening or seriously debilitating condition who cannot be treated with available approved for use medicines and who are unable to join a clinical trial
- be under review for approval or being studied in clinical trials
- have sufficient evidence of efficacy and safety.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Germany (cont.)

4. What is the process for requesting Expanded Access in Germany?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If a compassionate use program is in place and the company approves the request, the treating physician must inform the patient about potential risks and potential benefits of the investigational medicine.

Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is open in Germany for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

In a compassionate use program in Germany the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided until the medicine is approved for use and available to be prescribed by a treating physician within the German healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Germany (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Germany, the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Germany?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Germany?

Further information regarding the compassionate use pathway in Germany can be found on the PEI website and BfArM website. Please note that the information on these websites is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Greece

1. Is Expanded Access a potential option in Greece?

Yes, it is possible for an investigational medicine that is not approved for use in Greece to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Greece?

In Greece, there are two pathways for Expanded Access.

An investigational medicine can be provided for an individual patient in exceptional or very urgent situations and where all existing treatment options have been tried. The treating physician must apply to the National Organization for Medicines (EOF), the medicines regulatory agency in Greece, providing justification for use. If approved, the EOF issues a permit.

Alternatively, a program called an Early Access Program can be set-up voluntarily by the pharmaceutical company providing the investigational medicine. The program must be approved by the EOF, and then eligible patients can join without the need for the treating physician to submit an individual request to the EOF.

For an early access program to be set up, the investigational medicine must:

- be intended for patients with a life-threatening or serious condition and who cannot be treated with medicines that are approved for use in Greece and are unable to join a clinical trial
- be under review for approval or currently studied in clinical trials

Greece (cont.)

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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4. What is the process for requesting Expanded Access in Greece?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If an Early Access Program is not available, the physician must also gain approval to use the investigational medicine for their patient from the EOF.

If the company (and EOF, if necessary) approves the request, the treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

Greece (cont.)

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if an Early Access Program is approved by the EOF for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Greek healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Greece it can be made available through Expanded Access for a different condition if the key requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

Greece (cont.)

8. What are the key considerations for requesting Expanded Access when the patient does not live in Greece?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in Greece?

Further information regarding the Expanded Access pathways in Greece can be found on the EOF website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Hungary

1. Is Expanded Access a potential option in Hungary?

Yes, it is possible for an investigational medicine that is not approved for use in Hungary to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Hungary?

In Hungary, an investigational medicine can be provided in exceptional circumstances for a patient:

- with a life-threatening or serious condition and
- who cannot be treated satisfactorily with authorized medicines and
- who cannot be included in a clinical trial and
- who has provided their written informed consent following receipt of information about treatment with the investigational medicine, including information on the risks and potential benefits.

The investigational medicine must be under study in clinical trials or under review for approval and there must be sufficient evidence available from clinical trials supporting the potential effectiveness and safety of the investigational medicine.

The treating physician must have approval to use the investigational medicine for their patient from the pharmaceutical company and agreement that the company will provide the investigational medicine free of charge for the duration of treatment or until the medicine is approved for use and available in Hungary.

The treating physician must also gain approval from the National Center for Public Health and Pharmacy (NNGYK), the medicines regulatory agency in Hungary, for the use of the investigational medicine, prior to initiating treatment.

Hungary (cont.)

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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4. What is the process for requesting Expanded Access in Hungary?

The treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the company approves the request, the treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

The treating physician must then apply to the NNGYK, providing justification for the use of the investigational medicine. Requests are reviewed within 21 days. The NNGYK issues a permit to the health facility if the request is approved, and the investigational medicine can then be imported, and treatment initiated.

Hungary (cont.)

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Hungary for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

The investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it for the duration of treatment. If the medicine is approved for use and available to be prescribed by a treating physician within the Hungarian healthcare system patients will become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Hungary it can be made available through Expanded Access for a different condition if the key requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

Hungary (cont.)

8. What are the key considerations for requesting Expanded Access when the patient does not live in Hungary?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in Hungary?

Further information regarding the Expanded Access pathway in Hungary can be found on the NNGYK website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Ireland

1. Is Expanded Access a potential option in Ireland?

Yes, it is possible for an investigational medicine that is not approved for use in Ireland to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Ireland?

In certain circumstances, a patient can be prescribed an investigational medicine under the direct responsibility of the treating doctor, to fulfill the medical needs of their patient. Investigational medicines supplied outside of a clinical trial in Ireland are known as 'exempt medicinal products'.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating doctor is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

4. What is the process for requesting Expanded Access in Ireland?

The treating doctor must submit a request for the investigational medicine to the pharmaceutical company developing it. Approval is not required from the Health Products Regulatory Authority (HPRA), the medicines regulatory agency in Ireland.

If the company approves the request, the treating doctor informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

Ireland (cont.)

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating doctor. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Ireland for the relevant condition
- qualifications of the requesting doctor and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating doctor within the Irish healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Ireland the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with the treating doctor.

Ireland (cont.)

8. What are the key considerations for requesting Expanded Access when the patient does not live in Ireland?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating doctor is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating doctor.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

9. Where can I read more about Expanded Access in Ireland?

Further information regarding the exempt medicinal products pathway in Ireland can be found on the HPRA website. Please note that the information on the website is intended for healthcare professionals. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating doctor.

Italy

1. Is Expanded Access a potential option in Italy?

Yes, it is possible for an investigational medicine that is not approved for use in Italy to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Italy?

A patient with a life-threatening, rare, or serious condition may be treated with an investigational medicine if the patient has no alternative treatment options and is not eligible to participate in a clinical trial. This is known as 'compassionate use' in Italy.

For compassionate use, the investigational medicine must be under review for approval or currently being studied in clinical trials. There must also be sufficient evidence of potential efficacy and safety.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Italy (cont.)

4. What is the process for requesting Expanded Access in Italy?

The treating physician must submit a request for the investigational medicine to the pharmaceutical company.

The treating physician must also gain approval from their local Ethics Committee to use the investigational medicine for their patient. The Ethics Committee notifies the Italian Medicines Agency (AIFA), the medicines regulatory agency in Italy, of their decision.

If all approvals are obtained, the treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Italy for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

The investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Italian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Italy (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Italy it can be made available through Expanded Access for a different condition if the key requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Italy?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Italy?

Further information regarding the compassionate use pathway in Italy can be found on the AIFA website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Latvia

1. Is Expanded Access a potential option in Latvia?

Yes, it is possible for an investigational medicine that is not approved for use in Latvia to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Latvia?

For Expanded Access in Latvia, the investigational medicine must:

- be intended for patients with a life-threatening or serious condition who cannot be treated with available approved for use medicines and who are unable to join a clinical trial
- be under study in clinical trials or be under review for approval for use

The investigational medicine must be provided within a program called a 'compassionate use program', set up voluntarily by the pharmaceutical company developing it. The program requires approval from the State Agency of Medicines (SAM), the medicines regulatory agency in Latvia, before patients can enter.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Latvia (cont.)

4. What is the process for requesting Expanded Access in Latvia?

In all cases, the treating physician must submit a request to the pharmaceutical company developing the investigational medicine.

If an approved compassionate use program is in place and the request is approved by the pharmaceutical company, the company provides all the relevant information on the investigational medicine to the treating physician.

The treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is open in Latvia for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

In a compassionate use program in Latvia the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided until the medicine is approved for use and available to be prescribed by a treating physician within the Latvian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Latvia (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Latvia, the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Latvia?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Latvia?

Further information regarding the compassionate use pathway in Latvia can be found on the SAM website. Please note that the information on this website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Lithuania

1. Is Expanded Access a potential option in Lithuania?

Expanded Access has recently been incorporated into medicines law in Lithuania which will allow for a pharmaceutical company to provide access to an investigational medicine for a patient outside of a clinical trial in certain exceptional circumstances.

Further regulations are awaited to enable Expanded Access to operate in practice in Lithuania.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Patient guide

Luxembourg

1. Is Expanded Access a potential option in Luxembourg?

Yes, it is possible for an investigational medicine that is not approved for use in Luxembourg to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Luxembourg?

In Luxembourg, Expanded Access can be provided in certain exceptional circumstances.

The investigational medicine must be intended for a patient with a life-threatening or serious condition and who cannot be treated with medicines that are approved for use and available in Luxembourg. There must also be supporting clinical evidence of potential efficacy and safety of the investigational medicine for the intended use.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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4. What is the process for requesting Expanded Access in Luxembourg?

The treating physician must gain approval to use the investigational medicine for their eligible patient from the pharmaceutical company developing it and from the Ministry of Health, the central health authority in Luxembourg.

Luxembourg (cont.)

If the request is approved, the treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine to other patients in Luxembourg for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Luxembourg healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Luxembourg (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Luxembourg it can be made available through Expanded Access for a different condition if the key requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Luxembourg?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Luxembourg?

Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Malta

1. Is Expanded Access a potential option in Malta?

Yes, it is possible for an investigational medicine that is not approved for use in Malta to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Malta?

In Malta, Expanded Access can be provided for an individual patient when the investigational medicine is needed to treat a special medical need that cannot be met by medicines that are approved for use.

Expanded Access can also be provided in Malta within a program known as a compassionate use program (CUP). A pharmaceutical company may voluntarily initiate a CUP for an investigational medicine if it is being reviewed for approval or is being studied in clinical trials. A CUP can only be set up for patients with a life-threatening or serious condition with no alternative treatment options and cannot enter a clinical trial.

The company must have approval of the CUP from the Ministry for Health, the central health authority in Malta, before a patient can enter the program.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating doctor is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

Malta (cont.)

4. What is the process for requesting Expanded Access in Malta?

The treating doctor must submit a request for their eligible patient to the pharmaceutical company developing the investigational medicine.

If there is no approved CUP available, the doctor also submits a request for use of the investigational medicine to the Ministry for Health.

If the request is approved, the treating doctor informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is available in Malta for the investigational medicine for the relevant condition
- qualifications of the requesting doctor and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the timeframe that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating doctor within the Maltese healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Malta (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Malta it cannot usually be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating doctor.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Malta?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating doctor is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating doctor.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with their treating doctor.

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9. Where can I read more about Expanded Access in Malta?

Further information regarding Expanded Access in Malta can be found on the Ministry for Health website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating doctor.

Patient guide

The Netherlands

1. Is Expanded Access a potential option in the Netherlands?

Yes, it is possible for an investigational medicine that is not approved for use in the Netherlands to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in the Netherlands?

An investigational medicine may be requested when a patient is not able to use available and approved treatments and cannot join a clinical trial or a compassionate use programme (see below).

The doctor must confirm the above information in a written statement known as a Doctor's Declaration. Permission for supply of the investigational medicine is required for each patient from the Health and Youth Care Inspectorate (IGJ), part of the Ministry of Health, in the Netherlands.

Alternatively, the pharmaceutical company developing an investigational medicine may voluntarily initiate a compassionate use programme following approval from the Medicines Evaluation Board (MEB), the medicines regulatory agency in the Netherlands. A compassionate use programme can only be set up for patients with a serious condition for which no alternative medicines are available and who cannot enter a clinical trial. The investigational medicine must be under study in clinical trials or under review for approval. There must also be sufficient evidence supporting the use of the investigational medicine for the relevant condition.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating doctor is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

The Netherlands (cont.)

4. What is the process for requesting Expanded Access in the Netherlands?

The treating doctor must submit a request for their patient to the pharmaceutical company developing the investigational medicine.

If the company approves the request, a compassionate use programme is open and the patient meets the entry criteria, the patient can enter the programme.

If a compassionate use program is not available, the patient's doctor prepares a written statement (the 'Doctor's Declaration') providing justification for the use of the investigational medicine. A request, including the Doctor's Declaration, must then be submitted to the IGJ for approval. Requests are reviewed by the IGJ as quickly as possible, and approval is required before the patient can start treatment.

The treating doctor informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use programme is open in the Netherlands for the investigational medicine for the relevant condition
- qualifications of the requesting doctor and medical facility where the patient would be treated.

The Netherlands (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the timeframe that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating doctor within the Dutch healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in the Netherlands it cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating doctor.

8. What are the key considerations for requesting Expanded Access when the patient does not live in the Netherlands?

Due to a variety of circumstances, sometimes patients seek to gain, or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating doctor is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating doctor.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

The Netherlands (cont.)

9. Where can I read more about Expanded Access in the Netherlands?

Further information regarding the Expanded Access pathways in the Netherlands can be found on the IGJ website and the MEB website. Please note that the information on these websites is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating doctor.

Poland

1. Is Expanded Access a potential option in Poland?

Expanded Access is not currently included in Polish medicines law and therefore there are no defined procedures for a pharmaceutical company to allow access to an investigational medicine for a patient outside of a clinical trial.

In exceptional circumstances, the Ministry of Health in Poland may temporarily authorize the use of an investigational medicine in Poland for patients with a serious or life-threatening condition.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Patient guide

Portugal

1. Is Expanded Access a potential option in Portugal?

Yes, it is possible for an investigational medicine that is not approved for use in Portugal to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Portugal?

Expanded Access is possible when a treating physician considers an investigational medicine essential for the treatment of a patient and there are no suitable alternative medicines for the patient that are approved for use and available.

There must be sufficient supporting evidence of the potential efficacy and safety of the investigational medicine for the patient's condition.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

4. What is the process for requesting Expanded Access in Portugal?

The treating physician must submit a request for their patient to the pharmaceutical company developing the investigational medicine. The hospital must seek approval from the National Authority of Medicines and Health Products (Infarmed), the medicines regulatory agency in Portugal, for the use of the investigational medicine for the patient. This approval is known as an 'Exceptional Use Authorization'.

Portugal (cont.)

The treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine for other patients in Portugal for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the timeframe that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Portuguese healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Portugal (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Portugal it may be possible for it to be made available through Expanded Access for a different condition if certain requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Portugal?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Portugal?

Further information regarding the Exceptional Use Authorization pathway in Portugal can be found on the Infarmed website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Romania

1. Is Expanded Access a potential option in Romania?

Yes, it is possible for an investigational medicine that is not approved for use in Romania to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Romania?

For Expanded Access in Romania, the investigational medicine must be provided within a program called a 'compassionate use program'. A compassionate use program is set up voluntarily by the pharmaceutical company developing the investigational medicine and requires approval from the National Authority of Medicines and Medical Devices of Romania (NAMMDR), the medicines regulatory agency in Romania.

A compassionate use program can only be approved in Romania if the patients to be treated have a life-threatening or serious condition and cannot be treated with medicines that are approved for use and available in Romania. The investigational medicine must be undergoing clinical trials or under review for approval for use.

Approval is required for each patient to join the program from the NAMMDR and the pharmaceutical company. Only patients who meet the criteria for the compassionate use program and who cannot join a clinical trial can enter the program and be treated with the investigational medicine.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Romania (cont.)

4. What is the process for requesting Expanded Access in Romania?

The treating physician must submit a request for their patient to the pharmaceutical company developing the investigational medicine.

If a compassionate use program is in place and the patient meets the entry criteria, the company submits the physician's request to the NAMMDR for approval.

The treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is open in Romania for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

In a compassionate use program, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the timeframe that free of charge supply is available may vary, it is generally provided until the medicine is approved for use and available to be prescribed by a treating physician within the Romanian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Romania (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Romania it may be possible for it to be made available through Expanded Access for a different condition if certain requirements are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Romania?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Romania?

Further information regarding the compassionate use pathway in Romania can be found on the NAMMDR website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Slovakia

1. Is Expanded Access a potential option in Slovakia?

Yes, it is possible for an investigational medicine that is not approved for use in Slovakia to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Slovakia?

Expanded Access may be provided for a patient with a serious or life-threatening condition when there are no medicines in Slovakia that are approved for use that can be used to treat the patient. The investigational medicine must be under review for approval for use.

The pharmaceutical company must set up a 'treatment program' to supply the investigational medicine for Expanded Access and the program must be approved by the Ministry of Health in Slovakia.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a healthcare professional is permitted to submit a request for access to an investigational medicine for the patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Slovakia (cont.)

4. What is the process for requesting Expanded Access in Slovakia?

The treating physician submits a request to the pharmaceutical company developing the investigational medicine for their patient.

If the request is approved, the treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form.

The treating physician gains the necessary approvals from hospital management and Ethics Committee and submits a request to use the investigational medicine for their patient to the Ministry of Health. If the request is approved, the Ministry of Health issues an authorization, allowing the investigational medicine to be imported and for the patient to start treatment.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if there is a treatment program available in Slovakia for the investigational medicine for the patient's condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

Slovakia (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

In a treatment program, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the timeframe that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Slovakian healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Slovakia it can be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in Slovakia?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Slovakia (cont.)

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in Slovakia?

Any questions patients, families, or caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Slovenia

1. Is Expanded Access a potential option in Slovenia?

Yes, it is possible for an investigational medicine that is not approved for use in Slovenia to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Slovenia?

In Slovenia, Expanded Access may be available through a program known as a 'compassionate use program', initiated voluntarily by the company developing the investigational medicine.

A compassionate use program allows access to an investigational medicine for patients with a life-threatening or severe condition that cannot be treated with medicines that are approved for use and available in Slovenia. The investigational medicine must be under study in clinical trials or under review for approval for use.

A compassionate use program requires approval from the Agency for Medicinal Products and Medical Devices of the Republic of Slovenia (JAZMP), the medicines regulatory agency in Slovenia.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Slovenia (cont.)

4. What is the process for requesting Expanded Access in Slovenia?

The treating physician must submit a request for their patient to the pharmaceutical company developing the investigational medicine.

If a compassionate use program is available and the request is approved, the hospital gains approval from the national Ethics Committee in Slovenia, if required. The pharmaceutical company can then request approval for the hospital to join the program from JAZMP.

If the request is approved by JAZMP, the treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is approved by JAZMP for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

Slovenia (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

In a compassionate use program, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. Free of charge supply of the investigational medicine is provided until the program ends, and for at least another year afterwards, for the patients who were included in the program. Once the medicine is approved for use and available to be prescribed by a treating physician within the Slovenian healthcare system, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Slovenia it may be possible for it to be made available through Expanded Access for a different condition if certain conditions are met. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in Slovenia?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Slovenia (cont.)

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in Slovenia?

Further information regarding compassionate use programs in Slovenia can be found on the JAZMP website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

South Korea

1. Is Expanded Access a potential option in South Korea?

Yes, it is possible for an investigational medicine that is not approved for use in South Korea to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in South Korea?

In South Korea, to be considered for Expanded Access, a patient must have a serious or life-threatening condition, and not be able to be treated with available medicines. This is known as compassionate use.

There must be an ongoing or completed clinical trial with the investigational medicine in South Korea, and there must be sufficient evidence of potential efficacy and safety.

For compassionate use for an individual patient, the physician requires approval from the Ministry of Food and Drug Safety (MFDS), the medicines regulatory agency in South Korea, in advance of initiating treatment with the investigational medicine. In certain circumstances, compassionate use for an individual patient may be possible if there is no clinical trial with the investigational medicine in South Korea, so long as there is a clinical trial that is ongoing or completed in another country.

Alternatively, the pharmaceutical company developing the investigational medicine can apply to the MFDS for approval to treat multiple patients in a compassionate use program.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

South Korea (cont.)

4. What is the process for requesting Expanded Access in South Korea?

In all cases, the treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If a compassionate use program is in place and the company approves the physician's request, the health facility must gain approval from an Institutional Review Board (IRB) prior to initiating treatment of the patient with the investigational medicine.

If treatment is for an individual patient, when there is no compassionate use program in place, the treating physician must obtain a written letter from the pharmaceutical company confirming they agree to supply the investigational medicine. The physician must then apply to the MFDS for approval to use the investigational medicine for their patient. This application process can take up to 30 days for the MFDS to issue a decision.

The treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if a compassionate use program is approved in South Korea for the investigational medicine in the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

South Korea (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Korean healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in South Korea, the medicine cannot usually be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in South Korea?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

South Korea (cont.)

9. Where can I read more about Expanded Access in South Korea?

Further information regarding the compassionate use pathway in South Korea can be found on the MFDS website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Spain

1. Is Expanded Access a potential option in Spain?

Yes, it is possible for an investigational medicine that is not approved for use in Spain to be made available, if certain requirements are met

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2. What are the key requirements for Expanded Access in Spain?

In Spain, Expanded Access is possible for a patient with a life-threatening or serious condition who cannot be treated with medicines which are approved for use in Spain and who cannot enter a clinical trial. This is known as compassionate use.

The investigational medicine must be under study in clinical trials or be under review for approval for use. There must also be sufficient supporting evidence of the investigational medicine for the treatment of the patient's condition.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Spain (cont.)

4. What is the process for requesting Expanded Access in Spain?

The treating physician gains approval from their hospital management for the use of the investigational medicine and then submits a request for their patient to the pharmaceutical company.

If the request is approved, the physician applies to the AEMPS for approval to treat the patient with the investigational medicine. The AEMPS issues an authorization to import the investigational medicine if the request is approved.

The treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if the company is already providing access to the investigational medicine for other patients in Spain for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Spanish healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

Spain (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Spain it cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in Spain?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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9. Where can I read more about Expanded Access in Spain?

Further information regarding the compassionate use pathway in Spain can be found on the AEMPS website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Sweden

1. Is Expanded Access a potential option in Sweden?

Yes, it is possible for an investigational medicine that is not approved for use in Sweden to be made available, if certain requirements are met.

2. What are the key requirements for Expanded Access in Sweden?

There are two potential pathways for Expanded Access in Sweden, a compassionate use program (CUP) or a Special License.

The company developing an investigational medicine may voluntarily set up a CUP for patients with a serious or life-threatening condition who are unable to join a clinical trial and who cannot be treated with available and approved treatments. The investigational medicine must be currently studied in clinical trials or under review for approval. There must also be sufficient supporting evidence from clinical trials.

A CUP requires approval from the Swedish Medical Products Agency (MPA), the medicines regulatory agency, in Sweden.

Alternatively, if a patient cannot enter a CUP or a clinical trial and has a medical need that cannot be treated using medicines that are approved for use, it may be possible for a treating physician to treat the patient using an investigational medicine if the MPA grants a 'Special License'.

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a healthcare professional is permitted to submit a request for access to an investigational medicine for the patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Sweden (cont.)

4. What is the process for requesting Expanded Access in Sweden?

The treating physician submits a request for their patient to access the investigational medicine to the pharmaceutical company.

If the request is approved, and there is no CUP available, the hospital pharmacy must also apply to the MPA for a Special License. The treating physician must provide justification for use of the investigational medicine as part of the application. The MPA usually processes applications for a Special License within 7 working days but can take longer in some cases. Very urgent cases may be responded to the same day.

The treating physician informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, may be required to sign a consent form prior to beginning treatment with the investigational medicine.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if there is a CUP available in Sweden for the investigational medicine for the patient's condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

Sweden (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Swedish healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Sweden it cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in Sweden?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Sweden (cont.)

9. Where can I read more about Expanded Access in Sweden?

Further information regarding the Special License and the Compassionate Use pathways in Sweden can be found on the MPA website. Please note that the information on the website is intended for healthcare professionals and pharmaceutical companies. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

Switzerland

1. Is Expanded Access a potential option in Switzerland?

Yes, it is possible for an investigational medicine that is not approved for use in Switzerland to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in Switzerland?

If a clinical trial is being conducted in Switzerland, the pharmaceutical company can apply to Swissmedic, the medicines regulatory agency in Switzerland, for a temporary authorization to provide access to the investigational medicine to other patients who could not be included in the clinical trial. For a temporary authorization there must be no alternative approved medicines for the condition to be treated.

If there is no temporary authorization in place, it may be possible for a treating physician to import and treat a patient with an investigational medicine if the patient has no alternative treatment options and the physician completes a risk assessment.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Switzerland (cont.)

4. What is the process for requesting Expanded Access in Switzerland?

The treating physician must submit a request for the investigational medicine to the pharmaceutical company.

If the company approves the request, the treating physician must inform the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

If the treating physician is treating an individual patient when there is no temporary authorization in place, the treating physician must also complete and submit the conclusion of the risk assessment to their local cantonal authority. Once the cantonal authority has confirmed receipt, the physician can import the investigational medicine and the patient can be treated.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if there is a temporary authorization in place in Switzerland for the investigational medicine for the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

Switzerland (cont.)

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the Swiss healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in Switzerland the medicine cannot be made available through Expanded Access for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

8. What are the key considerations for requesting Expanded Access when the patient does not live in Switzerland?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

Switzerland (cont.)

9. Where can I read more about Expanded Access in Switzerland?

Further information regarding the temporary authorization pathway in Switzerland can be found on the Swissmedic website. Please note that the information on the website is intended for healthcare professionals. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Patient guide

United Kingdom (UK)

1. Is Expanded Access a potential option in the UK?

Yes, it is possible for an investigational medicine that is not approved for use in the UK to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in the UK?

An investigational medicine may be supplied for a patient with an unmet medical need, following a request from the treating doctor. Investigational medicines supplied in this way are known in the UK as 'Specials'. A Special can only be supplied if there are no other treatment options for the patient.

Alternatively, an investigational medicine can be supplied within an Early Access to Medicines Scheme (EAMS) for a group of patients with life-threatening or serious disease and with a clear unmet medical need. An EAMS is set-up voluntarily by the pharmaceutical company developing the investigational medicine and must be approved by the Medicines and Healthcare Products Regulatory Agency (MHRA), the medicines regulatory agency in the UK.

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3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating doctor is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

United Kingdom (cont.)

4. What is the process for requesting Expanded Access in the UK?

The treating doctor must submit a request for the investigational medicine to the pharmaceutical company developing it.

If the company approves the request, the treating doctor informs the patient about potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

If the investigational medicine is being supplied as a Special and is being imported from another country, the importing company must inform the MHRA of their intention to import, giving at least 28 days' notice.

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if an EAMS is open in the UK for the investigational medicine for the relevant condition
- qualifications of the requesting doctor and medical facility where the patient would be treated.

6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. If a company sets up an EAMS, the investigational medicine must be provided free of charge. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating doctor within the UK healthcare system. At that time, patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

United Kingdom (cont.)

7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in the UK the medicine cannot be made available through Expanded Access for a different condition unless there is an approved EAMS in place for the medicine in the relevant condition. Patients are encouraged to discuss all potential treatment options with the treating doctor.

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8. What are the key considerations for requesting Expanded Access when the patient does not live in the UK?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating doctor is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating doctor.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating doctor.

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9. Where can I read more about Expanded Access in the UK?

Further information regarding the EAMS and Specials pathways in the UK can be found on the MHRA website. Please note that the information on the website is intended for healthcare professionals, pharmaceutical companies, and medicines suppliers. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating doctor.

Patient guide

United States (U.S.)

1. Is Expanded Access a potential option in the U.S.?

Yes, it is possible for an investigational medicine that is not approved for use in the U.S. to be made available, if certain requirements are met.

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2. What are the key requirements for Expanded Access in the U.S.?

For Expanded Access in the U.S., it is required that the patient has a serious or life-threatening condition, the potential benefit of treatment with the investigational medicine justifies the risks, there are no comparable or satisfactory alternative therapies, and the patient cannot enter a clinical trial.

Expanded Access requires approval from the pharmaceutical company developing the investigational medicine and the U.S. Food and Drug Administration (FDA), the medicines regulatory agency in the U.S.

The pharmaceutical company may provide access to an investigational medicine for a particular condition through an **Expanded Access** protocol. The protocol describes which patients can receive the investigational medicine and how patients are treated. The company must gain approval of the protocol from the FDA. Under an Expanded Access protocol, following a request to the company by the treating physician, a patient who meets the defined entry criteria can be treated with the investigational medicine, as described in the protocol, without further FDA approval.

If an Expanded Access protocol is not in place, a treating physician must have approval from the FDA for the use of the investigational medicine for the individual patient.

For all cases of Expanded Access, approval from an Institutional Review Board (IRB) is required before treatment with the investigational medicine can begin, except in exceptional emergency situations.

United States (cont.)

3. Can a patient, family member, or caregiver submit a request to the regulatory agency or a pharmaceutical company?

Only a treating physician is permitted to submit a request for access to an investigational medicine for their patient. Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

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4. What is the process for requesting Expanded Access in the U.S.?

The treating physician must submit a request for their patient to the pharmaceutical company developing the investigational medicine.

If the request is approved by the company but there is no Expanded Access protocol, the treating physician must submit a single patient Expanded Access request to the FDA. The FDA has up to 30 days to review the application, but it is usually completed within a few days.

The treating physician must also apply to an IRB and gain approval for the Expanded Access use.

The treating physician must inform the patient about the potential risks and potential benefits of the investigational medicine. Additionally, the patient, or their legal representative, will be required to sign a consent form prior to beginning treatment with the investigational medicine.

For emergency cases, it is possible for a treating physician to apply to the FDA for Expanded Access under an urgent procedure.

United States (cont.)

5. What does a company consider when reviewing an Expanded Access request from a physician?

When considering a request, companies need various details from the treating physician. The time required for a company to review a request and provide a decision may vary.

Examples of considerations include:

- patient's medical history and current condition
- the policy or process at the company manufacturing the investigational medicine
- if an Expanded Access protocol is approved by the FDA for the investigational medicine for use in the relevant condition
- qualifications of the requesting physician and medical facility where the patient would be treated.

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6. Who pays for the investigational medicine and how long is it generally available?

Typically, the investigational medicine is provided free of charge by the pharmaceutical company developing and manufacturing it. While the amount of time that free of charge supply is available may vary, it is generally provided up until the medicine is approved for use and available to be prescribed by a treating physician within the U.S. healthcare system. At that time patients become responsible for payment of the medicine through their usual process to obtain prescription medicines.

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7. If a medicine has received approval for one condition, can Expanded Access be requested for a different condition?

Once a medicine has received approval for use and is available in the U.S., Expanded Access is not usually possible for a different condition. Patients are encouraged to discuss all potential treatment options with their treating physician.

United States (cont.)

8. What are the key considerations for requesting Expanded Access when the patient does not live in the U.S.?

Due to a variety of circumstances, sometimes patients seek to gain or continue access to an investigational medicine outside of their country of residence.

As in all cases of Expanded Access, a local treating physician is required to accept responsibility for their care and to agree to request access to the investigational medicine. The pharmaceutical company will also need to agree to provide the investigational medicine to the new treating physician.

It is important to understand that moving to another country does not guarantee access to an investigational medicine, even if the investigational medicine is available via Expanded Access in that country.

Patients, families, or caregivers are encouraged to discuss all potential treatment options with the treating physician.

9. Where can I read more about Expanded Access in the U.S.?

Further information regarding Expanded Access in the U.S. can be found on the FDA website and Reagan-Udall Foundation website. In addition, ClinicalTrials.gov provides details of open Expanded Access programs. Any questions patients, families, and caregivers may have regarding Expanded Access should be directed to the patient's treating physician.

Glossary

Agencia Española de Medicamentos y Productos Sanitarios (AEMPS)	The AEMPS is a state agency in Spain attached to the Ministry of Health. It is responsible for guaranteeing to society, from a public service perspective, the quality, safety, efficacy and correct information of medicines and health products, from their research to their use, in the interests of protecting and promoting human health, animal health and the environment. For more information: https://www.aemps.gob.es/la-aemps/quienes-somos/?lang=en
ANSM	The National Agency for the Safety of Medicines and Health Products is the public actor which, on behalf of the State, allows access to health products in France and which ensures their safety throughout their life cycle. For more information: https://www.ansm.sante.fr/L-ANSM/Une-agence-d-expertise/L-ANSM-agence-d-evaluation-d-expertise-et-de-decision/(offset)/0
BASG	BASG is the national authority for drugs, medical devices, blood and tissue in Austria. The BASG monitors - nationally and in concert with the European sister agencies - the drugs and medical devices that are already on the market with regard to their effectiveness, possible side effects, their production, transport and storage. For more information: https://www.basg.gv.at/ueber-uns
Bulgarian Drug Agency	The Bulgarian Drug Agency at the Ministry of Healthcare is defined as a body for the supervision of the quality, efficiency and safety of medicines. For more information: https://www.bda.bg/en/about-bda/history#from-nimp-to-bda
Clinical trial(s)	Interventional study (clinical trial) is a study in which participants are assigned to groups that receive one or more intervention/treatment (or no intervention) so that researchers can evaluate the effects of the interventions on biomedical or health-related outcomes. The assignments are determined by the study's protocol. Participants may receive diagnostic, therapeutic, or other types of interventions. For more information: https://www.clinicaltrials.gov/ct2/about-studies/glossary

Glossary (cont.)

ClinicalTrials.gov

ClinicalTrials.gov is an online database of publicly and privately supported clinical trials conducted around the world. Doctors and patients may consult this database when seeking access to an experimental drug, via either a clinical trial or pre-approval access. While ClinicalTrials.gov is operated by the United States National Institutes of Health (NIH), being listed in the database should not be seen as an endorsement by the NIH or the U.S. Food and Drug Administration (FDA) of the value of any agent or product.

Commercially available

A product that has received local health authority approval and the product is able to be prescribed.

Compassionate use

"Compassionate use" is access to investigational drugs that have not yet been approved for sale or use by the U.S. Food and Drug Administration (FDA) or relevant regulatory authority, such as Health Canada. The term is often used interchangeably with "expanded access" and "pre-approval access."

Often investigational drugs may be available to patients through clinical trials. Compassionate use is the provision of the drug to patients who are unable to participate in a clinical trial because of severity of illness or some other factor.

Sponsors decide whether to make their investigational drugs available to patients via compassionate use. Should a sponsor, often a private company, be willing to provide the investigational drug, the FDA must approve the planned use of that drug. FDA regulations specify two groups of people eligible for compassionate use: 1) those with life-threatening diseases or conditions for which "there is a reasonable likelihood that death will occur within a matter of months or in which premature death is likely without early treatment" and 2) those with serious diseases or conditions that have a "substantial impact on day-to-day functioning" (21 Code of Federal Regulations 312.300(b)). In most cases, patients who seek compassionate use must have exhausted all approved therapies for their condition and be unable to enroll in a clinical trial.

Glossary (cont.)

Danish Medicines Agency (DMA)	The Danish Medicines Agency authorises and inspects pharmaceutical companies and licenses medicinal products in the Danish market, monitors adverse reactions from medicinal products, authorises clinical trials, decides which medicines are eligible for reimbursement, monitors medical devices available in Denmark and supervises adverse incidents involving medical devices, appoints proprietary pharmacists, organises the pharmacy structure and supervises pharmacies and retailers. They perform most of their tasks in close cooperation with colleagues from regulatory authorities and organisations in the other EU countries. For more information: https://laegemiddelstyrelsen.dk/en/about/
Early Access to Medicines Scheme (EAMS)	The Early Access to Medicines Scheme (EAMS) in the United Kingdom aims to make promising new medicines available to patients sooner. It was set up in 2014 and is run by the Medicines and Healthcare Products Regulatory Agency (MHRA).
Eligibility criteria	Eligibility criteria are the key requirements for entry into a clinical study or other investigational medicine program such as expanded access. For more information: https://www.clinicaltrials.gov/ct2/about-studies/glossary
Ethics committee	Ethics committees are a group of individuals formed to protect the interests of patients and address moral issues. It normally includes a board member of the institution, a lay person, and an administrator. Most ethics committees work in an advisory capacity; they can help patients and families reach informed decisions and work with healthcare providers in order to make complex and difficult decisions.
Expanded Access	"Expanded access" (EA) is the U.S. Food and Drug Administration's (FDA) term for access outside clinical trials to investigational drugs that the agency has not yet approved. Expanded access is an umbrella term that applies to single patient requests and programs for groups, either intermediate-size or larger. Treatment of the patient, rather than collection of data, is the primary goal. This is also known as a "Group" or "Cohort" program.

Glossary (cont.)

Expanded Access Programs (EAPs)	EAPs are designed to permit larger groups of patients to access an investigational drug. For both single patient requests and EAPs, treatment of the patient, rather than collection of data, is the primary goal. However, in an EAP, data are frequently collected from patients enrolled in the program.
Federal Agency for Medicines and Health Products (FAMHP)	The FAMHP is the competent authority responsible for the quality, safety and efficacy of medicines and health products in Belgium. For more information: https://www.famhp.be/en/famhp
FIMEA	Operating under the Ministry of Social Affairs and Health, the Finnish Medicines Agency Fimea oversees and develops the pharmaceutical sector. For more information: https://www.fimea.fi/web/en/for_public/fimea-s-services-to-citizens
Form FDA 3926	Form 3926 is a U.S. Food and Drug Administration (FDA) form for use by physicians when submitting requests for expanded access to investigational drugs, including emergency requests. This form is designed specifically for single patient requests, not for EAPs. In 2016, this form was created as a shorter, streamlined alternative to Form FDA 1571, which still must be used for EAPs.
Health Authority	A Health Authority is a government agency that is responsible for national health services care in a particular area.
Health Canada	Health Canada is a federal institution that is part of the Health portfolio that is responsible for helping Canadians maintain and improve their health. It ensures that high-quality health services are accessible, and works to reduce health risks.

Glossary (cont.)

Individual request	Named patient requests based on an application made by the physician and/or local importer e.g., pharmacy or wholesaler.
Infarmed	National Authority for Medicines and Health Products, IP, abbreviated as Infarmed, is a public institute with a special regime, under the terms of the law, integrated in the indirect administration of the State, endowed with administrative, financial and own assets. Infarmed continues the duties of the Ministry of Health, under the supervision and supervision of the respective minister. For more information: https://www.infarmed.pt/web/infarmed/apresentacao
Institutional Review Board (IRB)	An IRB, sometimes referred to as a research ethics committee, is a committee charged with reviewing, approving, and monitoring biomedical and behavioral research involving humans. In the U.S., pre-approval access requires review and approval by an IRB. IRB review comes after the drug company agrees to provide access to an investigational product and the FDA reviews and accepts the proposed treatment plan.
Investigational new drug (IND)	Federal law requires that a drug or other therapeutic agent be approved for use before it can be transported and distributed in the U.S. An IND exemption is the means through which a sponsor obtains permission from the FDA to distribute the agent before it has this approval. Under an IND, sponsors may distribute an investigational drug to study it in clinical trials needed for approval. An IND must also be submitted to receive access to an investigational drug for compassionate use. There are four types of expanded access INDs: 1) individual patient expanded access, which allows compassionate use of a drug by a single patient; 2) Emergency Use IND, which allows the FDA to authorize the use of an investigational drug in an emergency situation, as it did during the 2014 Ebola outbreak; 3) Intermediate-Size Patient Population IND, which allows multiple patients to gain compassionate use access to an investigational drug, and 4) Treatment IND, which is submitted for the widespread use of investigational drugs showing promise in clinical testing for serious or immediately life-threatening conditions.

Glossary (cont.)

JAZMP	JAZMP of Slovenia's primary mission is to protect public health through the regulation and supervision of medicinal products, medical devices, blood, tissues and cells and associated activities in the private and public sector. For more information: https://www.jazmp.si/en/
Malta Medicines Authority	Malta's Medicines Authority's vision is to be a centre of excellence in advancing effective and innovative regulation and promoting quality and scientific rigour in the work we do. For more information: http://www.medicinesauthority.gov.mt/missionobjectives?l=1
Medical Product Agency (MPA)	The Medical Products Agency of Sweden is the responsible for regulation and surveillance of the development, manufacturing and sale of drugs and other medicinal products. For more information: https://www.lakemedelsverket.se/en
MHRA	The Medicines and Healthcare products Regulatory Agency regulates medicines, medical devices and blood components for transfusion in the UK.
Ministére-Direction de la Santé	The missions of the Ministére-Direction de la Santé, the Ministry of Health of Luxembourg, are the definition and application of government health policy, monitoring the application of health laws and regulations, supervision of health institutions and services. For more information: https://m3s.gouvernement.lu/en.html
Ministry of Health	The Ministry of Health of Poland is a government administration office that supports the minister of health. For more information: https://www.gov.pl/web/zdrowie/podstawowe-informacje
Named Patient Program	A program to provide access on a named patient (individual) or group/cohort basis.

Glossary (cont.)

National Agency for Medicines and Medical Devices of Romania (NAMMDR)	The National Agency for Medicines and Medical Devices (NAMMDR) is a public institution subordinated to the Ministry of Health of Romania. The NAMMDR mission is to help protect and promote public health. For more information: https://www.anm.ro/en/despre-institutie/despre-noi/
OGYÉI	OGYÉI is responsible for the tasks performed by the General Directorate of the GYEMSZI Institute of Pharmacy, the Directorate of Device Qualification and Hospital Technology, the Technology Evaluation Department, the GYEMSZI, the ÁNTSZ and the National Food and Nutrition Institute for Food and Nutrition. For more information: https://ogyei.gov.hu/engedelyezes_elotti_gyogyszeralkalmazas/
Phase 2 clinical trial(s)	Phase 2 Clinical Trial is a phase of research to describe clinical trials that gather preliminary data on whether a drug works in people who have a certain condition/disease (that is, the drug's effectiveness). For example, participants receiving the drug may be compared to similar participants receiving a different treatment, usually an inactive substance (called a placebo) or a different drug. Safety continues to be evaluated, and short-term adverse events are studied. For more information: https://www.clinicaltrials.gov/ct2/about-studies/glossary
Phase 3 clinical trial(s)	Phase 3 clinical trial is a phase of research to describe clinical trials that gather more information about a drug's safety and effectiveness by studying different populations and different dosages and by using the drug in combination with other drugs. These studies typically involve more participants. For more information: https://www.clinicaltrials.gov/ct2/about-studies/glossary

Glossary (cont.)

Pre-approval access (PAA)	PAA is an umbrella term encompassing access to investigational medicines, such as expanded access programs and compassionate use. PAA refers to any use of unapproved drugs outside of clinical trials, particularly if the intent is therapeutic rather than to gain data (research).
Single patient request (SPR)	Investigational access request outside of a clinical trial for one patient outside of an expanded access program.
Sponsor	A sponsor is the person or entity that takes responsibility for and initiates a clinical trial of an investigational agent. In the context of pre-approval access, the sponsor is typically a pharmaceutical or biotech company.
State Agency of Medicines (SAM) Estonia	State Agency of Medicines is a governmental body under the Ministry of Social Affairs in Estonia. Its main responsibility is the protection and promotion of public and animal health, through the supervision of medicines for human and veterinary use. For more information: https://www.ravimiamet.ee/en
State Agency of Medicines (SAM) Latvia	The State Agency of Medicines of Latvia (SAMLV) is a State institution under the supervision of the Ministry of Health of the Republic of Latvia. The operational objective of SAMLV is to implement local and international pharmaceutical legislation in order to ensure that the products (medicines, medical devices, blood, cells, tissues and organs) used in healthcare, as well as the involved companies and their activities comply with certain requirements. For more information: https://www.zva.gov.lv/en/about-us/about-agency

Glossary (cont.)

State Medicines Control Agency of Lithuania	The State Medicines Control Agency (SMCA) is a governmental body of the Republic of Lithuania with headquarters in Vilnius. Its main responsibility is the protection of public health, through the evaluation and supervision of medicines for human use. For more information: https://www.vvkt.lt/index.php?2380224066
Treating physician	Treating physician is the doctor who is providing medical treatment for a condition specific to expanded access requests.