Ireland

Agalsidase beta
home infusion therapy:

Manual for patients with Fabry disease who receive home infusion of agalsidase beta

The processes presented in this document serve as overall guidance but are subject to local medical practice and national rules and regulations.

If you get any side effects talk to your doctor, pharmacist or nurse. This includes any possible side effects not listed in the package leaflet. You can also report side effects directly to HPRA Pharmacovigilance, website: www.hpra.ie

By reporting side affects you can help provide more information on the safety of this medicine. Side effects should also be reported to Sanofi: Tel: 01 403 5600 e-mail: IEPharmacovigilance@sanofi.com

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Read all of this information carefully before you start home infusion.

This patient manual contains important safety information you need to be aware of when receiving treatment with agalsidase beta.

You should also have received the logbook from your treating physician with this patient manual.

- Keep this information in an easily accessible place; you may need to read it again.
- If you have further questions, ask your treating physician.
- This medicine has been prescribed for you. Do not pass it on to others even if their symptoms are the same as yours as it may harm them.
- If you experience any side effects, you and/or your caregiver must notify your treating physician or the healthcare professional (HCP) who administers your infusion.
- Please refer to the package leaflet for further information, this can also be found at www.medicines.ie.

1. YOUR DISEASE, TREATMENT AND HOME INFUSION

Together with your treating physician, you have decided to start home infusion therapy with agalsidase beta. **The objective of this document is to provide you with guidance on how to safely receive agalsidase beta at home.** The processes presented in this document serve as overall guidance but are subject to local medical practice and national rules and regulations. Your treating physician will provide you with the details that are applicable to your situation.

1.1 Fabry Disease and Treatment

Patients with Fabry disease have low or absent levels of an enzyme called alpha-galactosidase A. This enzyme is normally responsible for the breakdown of a fatty substance (globotriaosylceramide) and, as a result, abnormal deposits of this substance develop in blood vessel walls and other tissues throughout the body.

The main presenting childhood symptoms of Fabry disease in males include episodes of pain and burning sensations in the hands and feet, gastro-intestinal symptoms, skin rash and a decreased ability to sweat. Disease manifestations in adulthood are generally dominated by cardiac, renal and/or neurologic symptoms. In females, the course of the disease is variable, frequently - but not always - less severe than in affected males.

Agalsidase beta is an artificially produced enzyme which is intended to replace the natural enzyme alpha-galactosidase A that is lacking or not active enough in patients with Fabry disease. Agalsidase beta is used for the long-term treatment of patients who have a confirmed diagnosis of Fabry disease.

Refer to the agalsidase beta package leaflet for additional information.

1.2 Home Infusion

Currently, in some countries, people suffering from Fabry disease and treated with agalsidase beta receive their infusions at home. **The decision to receive home treatment should be made by you and your treating physician after initial infusions at the hospital to make sure you have no problems with the infusion. Your treating physician will arrange for all necessary treatment and equipment to be provided.**

An appropriately trained healthcare professional (HCP) (e.g. an infusion nurse) will teach and assist you and/or your caregiver(s) in the beginning to ensure optimal treatment. The HCP will teach you how to prepare and administer the infusion, how to manage side effects that may occur during or after the infusion and how to use the Logbook. The level of support required for home infusion will be discussed and agreed by you and/or your caregiver(s) and your treating physician. Should you prefer additional support for your infusion at home (after agreement of your treating physician), the HCP can provide further assistance.

Note: The dose and rate of the infusion while at home must follow the guidelines provided by your treating physician as noted in the Logbook and must not be changed without the prior agreement of your treating physician and supervision of the HCP.

1.3 Hypersensitivity reactions

Hypersensitivity reactions including severe life-threatening allergic reactions ('anaphylactoid reactions') have been reported in some patients. Signs and symptoms of these reactions include: localised swelling of the face, mouth and throat, narrowing of breathing airways, low blood pressure, hives, difficulty swallowing, rash, trouble breathing, flushing, chest discomfort, itching and nasal congestion.

Pre-treatment and Emergency Treatment

- If necessary, **your treating physician will prescribe pre-treatment medication to prevent such reactions from occurring.** Your treating physician will include the information on this medication in the Logbook.
- Your treating physician **will prescribe medication to respond to an emergency situation,** if necessary. Your treating physician will include the information on this medication in the Logbook. This emergency medication should be available during the infusions at home.

In the event that you experience signs and symptoms of a hypersensitivity reaction or do not feel well due to the medication during the home infusion or shortly after the infusion, you must immediately stop the medication. Immediately contact the treating physician, his/her medical designate, and/or the country-specific national emergency number (see instructions in the Logbook).

Your treating physician may decide to lower the infusion rate and subsequent infusions may need to occur in a clinical setting to prevent such reactions occurring again.

For the full list of all side effects reported with agalsidase beta, see the package leaflet available on www.medicines.ie

Any side effects must also be recorded in the Logbook.

If you become aware that a mistake was made in the preparation and/or administration of agalsidase beta, please contact the HCP or the treating physician to determine appropriate action before starting or continuing with the infusion. If you feel the treatment is not efficacious, please contact your treating physician.

2. THE LOGBOOK

- **You have been provided with a Logbook by your physician.** This will serve as a **means of communication** for everyone involved in administering agalsidase beta at home.
- The Logbook **must be kept at your home** and will be kept up to date by you, your caregiver(s), your treating physician and/or the HCP administering the infusion.
- **The prescribed dose and infusion rate of agalsidase beta as stated in the Logbook should be strictly followed.** The treating physician is responsible for describing the dose and the infusion rate, as well as any changes.
- **Each administration of agalsidase beta at home should be recorded in the Logbook.**
- You and/or your caregiver(s) **must take the Logbook along to the hospital** at each appointment for a check-up and bring it home afterwards.
- **The HCP administering the infusion records the findings and actions from the initial interview** and you, your caregiver(s) or the HCP notes all relevant information from subsequent visits in the Logbook.

In the Logbook, the treating physician must clearly state what has to be done and administered in the event of an infusion side effect. In case of any reaction to an infusion, the infusion needs to be stopped.

- Any infusion-associated side effect and/or medication error should be recorded in the Logbook.

3. TRAINING ON PREPARATION AND ADMINISTRATION OF AGALSIDASE BETA FOR PATIENTS WHO SELF-ADMINISTER

The initial instructions will be given at the hospital. The level of support required for home infusion will be discussed and agreed by you and/or your caregiver(s) and your treating physician.

- **Your treating physician is responsible for the organisation of the home infusion** and needs to agree upon the home infusion procedure.
- **Should you prefer to carry out the procedure yourself or with the assistance of your caregiver(s), you and/or your caregiver(s) will receive training from the HCP.** The HCP will explain and demonstrate the complete infusion procedure to you and/or your caregiver(s), including training in hand hygiene, proper disinfection and aseptic handling when preparing the infusion.
- **At subsequent visits, the HCP will be present to assist, if required,** until you and/or your caregiver(s) feel confident with the entire infusion procedure.
- While preparing and administering agalsidase beta, the procedures described in the package leaflet must be closely followed and, as described in this manual, must be adhered to.
- **Each administration of agalsidase beta should be recorded in the Logbook.**
- **The infusion should always be administered in the presence of an adult** knowledgeable about the infusion procedures and adequately trained on how to handle in cases of an infusion-associated reaction and medication errors (as assessed by the treating physician or HCP).

4. HOW DO I PREPARE AND ADMINISTER AGALSIDASE BETA?

4.1 Supplies

Supplied by the hospital/pharmacy to you or to a third party, and as prescribed by the treating physician.

- Vials of agalsidase beta (5 mg or 35 mg per vial); must be stored in a clean refrigerator at a temperature between +2°C and +8°C.
- Sterile water for injection to reconstitute agalsidase beta.
- NaCl 0.9% solution, 2 x 250 ml for IV administration.
- NaCl 0.9% solution, 2 x 50 ml to flush infusion line pre- and post-infusion.
- Chlorhexidine 0.5% in alcohol 70% (antiseptic solution).
- Appropriate number of 2 ml, 10 ml and 50 ml syringes depending upon dose of agalsidase beta.
- 3 x sterile hypodermic needles (1.1 x 40 mm).
- 1 x infusion needle.
- In-line low protein-binding 0.2 micron filter.
- Infusion-administration set (infusion line).
- Tape.
- Sterile skin cleansing swabs.
- Sharps bin.
- Hand wash.
- Tourniquet.
- Additional requisites if using a venous access device:
  - Heparin.
  - NaCl 0.9% solution.
  - Needles.
  - Syringes.
  - Dressing pack.
  - Sterile gloves.
  - Gripper needle.
- Pre-infusion treatment medication (if applicable; see Logbook for details provided by your treating physician).
- Emergency medication (See Logbook for instructions by treating physician).

4.2 Preparation

NOTE: The instructions for use (reconstitution, dilution and administration) can be found in the package leaflet available on www.medicines.ie. A detailed description is provided below.

1. Prepare a clean work area and lay out the supplies.
2. The vials of agalsidase beta must be removed from the refrigerator to reach room temperature approximately 30 minutes before preparation.
3. Check the expiry date printed on the bottom of the vial pack (do not use agalsidase beta after the labelled expiry date).
4. Verify if the number of vials received is correct.
5. Prepare only the number of vials required for one infusion.
6. Each vial is intended for single use only.

Note: The storage instructions as described in the instructions for use in the package leaflet must be followed carefully.



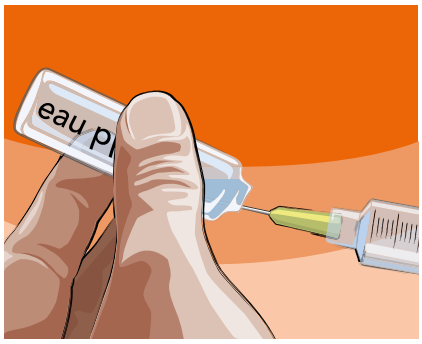
4.2 STEP 1: Preparation of the materials

4.3 Reconstituting agalsidase beta

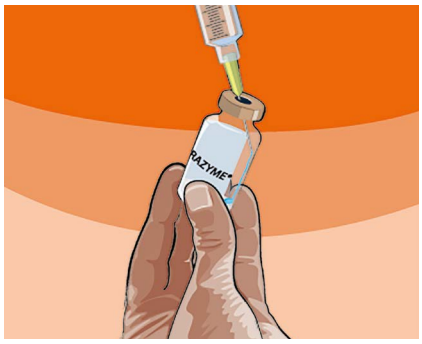
1. Remove the flip-off cap from the agalsidase beta vial.
2. Disinfect the rubber stopper of the agalsidase beta vial with chlorhexidine and allow to air dry.
3. Open the sterile water for injection.
4. Draw the required amount (ml) of sterile water into the syringe.
   - For 35 mg vials, reconstitute each vial with 7.2 ml water for injection.
   - For 5 mg vials, reconstitute each vial with 1.1 ml water for injection.
5. Avoid forcefully ejecting the water for injection from the syringe onto the powder, to minimise foaming. This should be done by slow drop-wise addition of the water for injection down the inside of the vial. Roll and tilt each vial gently. Do not invert, swirl or shake the vial.
6. Repeat the process for more agalsidase beta vials if required.
7. Small bubbles may appear after the mixing.
8. Let the solution settle for a few minutes to allow any bubbles present to disappear and to ensure that the powder is properly reconstituted.
9. After reconstitution, agalsidase beta must be inspected visually before use. The reconstituted solution must be a clear, colourless liquid and free from foreign matter. Because this is a protein solution, slight flocculation/cloudiness (in the form of thin translucent fibres) may occur occasionally after dilution.
10. If you notice any foreign matter or discolouration of the liquid, do not use the product and contact the HCP and/or treating physician.
11. It is recommended that the vials be diluted promptly after reconstitution, to minimise protein particle formation over time.
12. Any unused product or waste material must be disposed of in accordance with local requirements.



4.3 STEP 2: Disinfect the vial



4.3 STEP 4: Draw the required amount of sterile water into the syringe



4.3 STEP 5: Avoid forcefully ejecting the water for injections from the syringe

4.4 Dilution

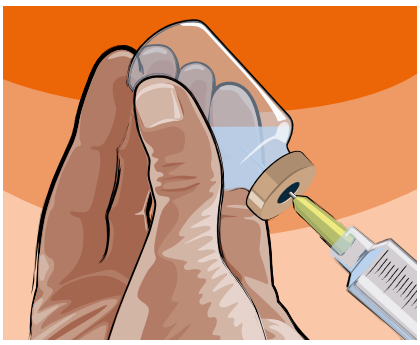
1. Disinfect the cap/opening of 1 or 2 bags of NaCl 0.9% solution using chlorhexidine and allow to air dry.
2. The volume of reconstituted agalsidase beta solution must be the same as the prescribed volume in the Logbook.
3. Insert the needle in the cap of the infusion bag and slowly withdraw a volume of NaCl 0.9% solution, equivalent to the volume of the reconstituted agalsidase beta solution to be added.

   For instance, if the prescribed reconstituted volume is 14 ml, remove 14 ml of NaCl solution from the bag of NaCl solution. Never remove more than half the content of the bag of NaCl solution to ensure that at least half the diluted solution consists of NaCl solution.

4. Remove the airspace within the infusion bag by withdrawing the air into a 50 ml syringe.
5. Slowly withdraw the reconstituted solution from each vial up to the total volume required. At the point when these quantities are withdrawn, the reconstituted product should not contain any foam.
6. Gently inject the total volume of the reconstituted agalsidase beta solution directly into the NaCl 0.9% solution. Do not inject the solution into any remaining airspace.
7. Carefully mix this agalsidase beta solution by gently inverting or lightly massaging the infusion bag. Do not shake or excessively agitate the infusion bag.
8. Determine the total volume of sodium chloride 0.9% solution for infusion as detailed in the Logbook.
9. The diluted solution should be filtered through an in-line low protein-binding 0.2 micron filter during administration.



4.4 STEP 3: Slowly withdraw the required volume of NaCl 0.9% solution, equivalent to the volume of the reconstituted agalsidase beta



4.4 STEP 5: Slowly withdraw the reconstituted solution from each vial up to the total volume required



4.4 STEP 5: The reconstituted product should not contain any foam

4.5 Administration

4.5.1 Filling the infusion line

1. Remove the infusion system from the package and close it using the roller clamp. Connect the in-line filter to the infusion line.
2. Connect the spike in the NaCl 0.9% solution bag that does not contain agalsidase beta and fill the infusion system by holding the drip chamber upside down and opening the clamp.
3. Fill the entire system, remove any air bubbles that may be present and close the roller clamp.
4. Connect the infusion bag containing agalsidase beta to the y-system. Keep the clamp closed.

4.5.2 Inserting the needle in the vein

In case of self-infusion, the adult person present during the infusion session should have been adequately trained (by the HCP, treating physician, or his/her medical designate) on the technique of needle insertion.

1. Ensure that some strips of tape are hanging ready for use and that the start of the infusion system is within reach. Place the chlorhexidine solution close by, along with some gauzes.
2. Remove the needle from the packaging.
3. Sit down and rest one arm on the table (preferably on a clean cloth).
4. Apply the tourniquet, look for an appropriate vein, and disinfect the area where the needle is to be inserted and allow it to dry.
5. Pull the skin tight and insert the needle (with its eye facing upward) at a slight angle through the skin and into the vein. When the needle has entered the vein, a 'flash' of blood will be visible at the start of the tubing.
6. Insert the needle approximately 0.5 cm in the vein to ensure that it does not immediately pop out again. Use tape to keep the needle into place. Connect the system with filter to the needle.
7. Remove the tourniquet; the tube will now fill up with blood. If this does not happen, the needle is not positioned correctly in the vein. The process must then be repeated using a new needle. Open the clamp for NaCl 0.9% solution.
8. Adjust the infusion rate according to the prescription (see the Logbook) and open the valve. Sit down and relax while the infusion takes place. Keep the Logbook close in case information on emergency procedures are needed.

4.5.3 Administration

- From a microbiological point of view, the product should be used immediately. If not used immediately, in-use storage and conditions are the responsibility of the user. The product diluted in NaCl 0.9% solution will retain chemical stability up to 24 hours if stored at a temperature between 2°C and 8°C and away from light.
- The agalsidase beta dose, infusion rate, as well as any changes, will be determined by the treating physician. The treatment must not be altered in the home setting, unless medically warranted at the discretion of the treating physician.
- After the agalsidase beta infusion has been completed, the system is flushed with NaCl 0.9% solution at the same rate, and the needle is then removed.

4.5.4 Preparation of the agalsidase beta infusion in case of a central venous access device

When you have a venous access device for the delivery of agalsidase beta, you and/or your caregiver(s) will be shown how to care for the device by the HCP, if this has not already been demonstrated during hospital-based infusions.

Proper care of a venous access device involves regular irrigation with a drug called heparin to prevent clotting and attention to a sterile technique to keep the device free of infection. The following steps are necessary:

- When in use, cover site with transparent occlusive dressing. No dressing required when not in use.
- Flush with 5 ml NaCl 0.9% solution before and after each use.
- Flush with 5 ml heparin (100 U/ml) after each use.