Fabrazyme® (agalsidase beta)
Home Infusion Therapy:

Manual for patients with Fabry Disease who receive home infusion of Fabrazyme (agalsidase beta)

VERSION NO. 2.0

Please report side effects to the MHRA through the Yellow Card scheme. You can report via:

- the Yellow Card website www.mhra.gov.uk/yellowcard
- the free Yellow Card app available from the Apple App Store or Google Play Store

Alternatively, you can report a suspected side effects to the Yellow Card scheme by calling 0800 731 6789 for free, Monday to Friday between 9am and 5pm. You can leave a message outside of these hours. When reporting, please provide as much information as possible. By reporting side effects, you can help provide more information on the safety of this medicine.

Side effects can also be reported to Sanofi: Tel: 0800 0902314. email: **UK-drugsafety@sanofi.com**

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

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

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

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 infusion nurse.
- For further information please refer to the package leaflet provided with your medicine and available online at www.medicines.org.uk/emc

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1. YOUR DISEASE, TREATMENT AND HOME INFUSION

You and your doctor have decided that you should start your Fabrazyme (agalsidase beta) treatment at home. This booklet will help you understand how to do this safely.

This guide gives you the main steps to follow. Your doctor will tell you exactly what you need to do for your situation.

1.1 What is Fabry Disease and How Do We Treat It?

What happens in Fabry disease?

People with Fabry disease don't have enough of something called an enzyme. Think of enzymes like tiny workers in your body. This particular worker is called alpha-galactosidase A.

This enzyme's job is to break down a fatty substance in your body. When you don't have enough of this enzyme, the fatty substance builds up. It gets stuck in your blood vessels and other parts of your body.

What symptoms might you have? If you're a boy or man with Fabry disease, you might have:

- Pain and burning feelings in your hands and feet
- Stomach problems
- Skin rashes
- Trouble sweating

When you get older, Fabry disease can affect your:

- Heart
- Kidneys
- Brain and nerves

If you're a girl or woman, your symptoms might be different. They're often milder than in boys and men, but not always.

How does your medicine help? Your medicine (Fabrazyme (agalsidase beta)) is a man-made version of the enzyme your body needs. It does the job that your natural enzyme can't do properly.

You'll need to take this medicine for a long time to help manage your Fabry disease.

Want to know more? You can read the medicine leaflet that comes with your Fabrazyme (agalsidase beta) for more information.

Refer to the Fabrazyme (agalsidase beta) Package Leaflet for additional information.

1.2 Getting Your Treatment at Home

What is home treatment?

In many places, people with Fabry disease can now get their Fabrazyme (agalsidase beta) treatment at home instead of going to the hospital. Before you start home treatment, you'll have a few treatments at the hospital first. This helps make sure the medicine works well for you without causing problems.

Why is home treatment helpful? Getting your treatment at home can make life easier for you:

- You can be comfortable in your own home
- You don't need to travel to the hospital
- You can plan treatments around school or activities
- You can fit treatments around family time
- You have more freedom with timing

Who is in charge of your home treatment? Your doctor is still in charge of your treatment, even when it happens at home. Your doctor will:

- Decide if home treatment is right for you
- Make sure you have all the information you need
- Check that your treatment is safe

Who will help you? A specially trained nurse will:

- Teach you and your helper how to do the treatment
- Show you all the steps
- Help you in the beginning
- Be there if you need extra support

You, your helper, and your doctor will talk about how much help you need. If you want more help at home, the nurse can give you extra support (if your doctor agrees).

⚠ **Important!** The amount of medicine and how fast it goes into your body must follow what your doctor has written in your Logbook. Never change these without talking to your doctor first and having the nurse supervise.

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1.3 Staying Safe During Treatment (Side effects and what to do if something goes wrong)

Can this medicine cause side effects?

Like all medicines, Fabrazyme (agalsidase beta) can sometimes cause side effects. But not everyone gets them.

Most side effects happen during the treatment or shortly after. These are called "infusion reactions."

In rare cases, some people have had serious allergic reactions. If this happens to you, tell your doctor straight away.

What are the most common side effects? More than 1 in 10 people may have:

- Chills or feeling cold
- Fever
- Feeling sick or being sick
- Headache
- Burning or tingling feelings on the skin

If you get these, your doctor might:

- Slow down how fast the medicine goes in
- Give you other medicines to help prevent these feelings

For a full list of side effects, read the medicine leaflet that comes with your Fabrazyme (agalsidase beta).

⚠️ What to do if you feel unwell

If you feel unwell during or just after your treatment:

1. Stop the treatment straight away
2. Call for help immediately – contact your doctor, their team, or emergency services (check your Logbook for numbers)
3. You may need to have your next treatments at the hospital instead of at home

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Keep a record and write down any side effects or symptoms in your Logbook.

What if a mistake is made? If you think something went wrong when preparing or giving the medicine:

- Stop – don't start or continue the treatment
- Call your nurse or doctor to find out what to do

What if you don't think the medicine is working? If you feel your treatment isn't helping, talk to your doctor about it.

2. ORGANISATION OF TREATMENT AT HOME

Your doctor will help set up your home treatment and will be in charge of it. This guide gives you the main steps, but your doctor will tell you exactly what to do for your situation.

2.1 What You Need to Know and Do

Before starting home treatment:

Your doctor will talk to you and your helper about:

- How the treatment works
- Any risks or problems that might happen
- What to do if you need medical help at home

You and your helper should:

- Understand Fabry disease
- Know how to spot side effects
- Know what to do if side effects happen

Your home needs to have:

- A clean area for treatment
- Electricity and water
- A telephone
- A refrigerator to keep your medicine cold
- Space to store your medicine and supplies

Safety rules:

- An adult who knows what to do must always be with you during treatment
- This could be a nurse or, if you do the treatment yourself, another trained adult who knows what to do if problems happen

You need to:

- Be well enough (physically and mentally) to have treatment at home
- Have veins that are easy to find for the needle

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- If you have a special device for your veins (central venous access device), you need to know how to use it
- Agree with your helper that you want treatment at home
- Be properly trained in how to prepare and give your medicine

2.2 What Your Doctor Does

Your doctor is in charge of:

- Starting all the paperwork so everyone else (the nurse, you, your helper, and the pharmacy) can do their jobs

Your doctor decides:

- How much medicine you need
- How fast the medicine should go into your body
- If you need other medicines before your treatment
- What emergency medicines you might need
- All of this information goes in your Logbook

If anything changes:

- Your doctor will tell you and your helper clearly
- The changes will be written in your Logbook

Your doctor makes sure:

- Your home treatment is safe
- You're ready for home treatment
- You have all the right information
- Everything is written down properly

Your doctor sets up:

- Phone numbers to call if you need help quickly
- These numbers go in your Logbook

Your doctor and nurse work together to:

- Plan when your treatments happen
- Check how your treatments are going

2.3 Pharmacy and Infusion Equipment

- Treatment and all necessary equipment will be provided according to local arrangements and regulations.

2.4 What Your Nurse Does

Your nurse helps plan your care:

- Works with you, your helper, and your doctor to decide how much support you need at home
- Helps organise your home treatment

Your nurse is trained to:

- Give medicines through a drip (into your vein)
- Give Fabrazyme (agalsidase beta) safely
- Spot side effects, including serious allergic reactions
- Know what to do if problems happen

During your treatment, your nurse will:

- Follow the steps in this booklet carefully
- Give you the exact amount of medicine your doctor has written in your Logbook
- Give the medicine at the right speed
- Write down each treatment in your Logbook

Your nurse and doctor work together to:

- Plan when your treatments happen
- Check how your treatments are going

⚠️ If you have a reaction during treatment

Your nurse will:

1. Stop the treatment straight away
2. Call your doctor or emergency services (using the numbers in your Logbook)

If you have a reaction just after your treatment finishes, your nurse will also call for help immediately.

Any reactions will be written down in your Logbook.

2.5 Extra Medicines You Might Need

Medicine before your treatment: If you need it, your doctor might give you medicine to take before your Fabrazyme (agalsidase beta) treatment. This can help prevent side effects.

If you need this medicine, your doctor will write the details in your Logbook.

Emergency medicine: Your doctor might also give you emergency medicine to keep at home. This is medicine you would only use if you have a serious reaction during treatment.

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If you have emergency medicine:

- Keep it with you during every treatment
- The details about when and how to use it will be in your Logbook
- Only use it when your Logbook or doctor tells you to

Remember: All information about these medicines will be written clearly in your Logbook.

2.6 Your Logbook

What is your Logbook? Your doctor has given you a special book called a Logbook. This book helps everyone involved in your care talk to each other and keep track of your treatment.

Where do you keep it?

- Keep your Logbook at home
- Everyone will write in it: you, your helper, your doctor, and your nurse

What's written in your Logbook?

- How much medicine you need
- How fast the medicine should go in
- Any changes your doctor makes
- You must follow exactly what's written - don't change anything without asking your doctor first

What do you write in it?

- Every time you have treatment at home
- Any side effects you have
- Any mistakes that happen
- Any other important information about your treatment

Take it with you:

- Bring your Logbook to every hospital appointment
- Your doctor will check it and may write new instructions
- Take it home again after each visit

Emergency information: Your Logbook will tell you:

- What to do if you have side effects
- What medicines to take in an emergency
- Who to call for help
- **Remember: if you have any reaction, stop the treatment right away**

Why is it important? Your Logbook keeps everyone informed about your care and helps keep you safe.

3. LEARNING HOW TO PREPARE AND GIVE YOUR MEDICINE

First steps: You'll learn how to do your treatment at the hospital first. You, your helper, and your doctor will talk about how much help you'll need at home.

Who's in charge: Your doctor is responsible for setting up your home treatment and must agree to the plan.

Learning to do it yourself: If you want to do the treatment yourself (or have your helper do it):

- Your nurse will teach you everything you need to know
- You'll learn how to:
- Wash your hands properly
- Clean everything correctly
- Keep everything germ-free when preparing your medicine

Getting comfortable:

- Your nurse will visit you at home to help at first
- They'll keep helping until you and your helper feel confident doing it on your own
- You can always ask for more help if you need it

⚠️ Important rules:

- Always follow the steps in this booklet and in the medicine leaflet exactly
- Write down each treatment in your Logbook
- Always have a trained adult with you during treatment
- This adult must know what to do if you have a reaction or if a mistake happens

Remember: Learning to do your treatment takes time. Don't worry if you don't feel confident right away - your nurse will help until you're ready.

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4. HOW TO PREPARE AND GIVE YOUR MEDICINE

4.1 What You'll Need

The hospital or pharmacy will give you everything you need. Your doctor decides what supplies you get.

Your medicine:

- Bottles (vials) of Fabrazyme (agalsidase beta) - either 5 mg or 35 mg size
- Keep in the fridge at 2°C to 8°C (cold but not frozen)

Liquids you'll need:

- Sterile water to mix with your medicine
- Salt water solution (NaCl 0.9%) - 2 large bottles (250 ml each) for your treatment
- Salt water solution (NaCl 0.9%) - 2 small bottles (50 ml each) to clean the tube before and after

Cleaning supplies:

- Antiseptic solution
- Sterile wipes to clean your skin
- Hand wash

Equipment:

- Syringes (different sizes: 2 ml, 10 ml, and 50 ml) - the number depends on your dose
- Needles (3 sterile needles)
- 1 needle for the treatment
- A special filter
- Tubing (infusion line)
- Tape to hold the needle in place
- A tourniquet (band to help find your vein)
- A sharps bin (special container for used needles)

If you have a special vein device, you'll also need:

- Heparin (medicine to prevent clotting)
- Extra salt water solution
- Extra needles and syringes
- Dressing pack

- Sterile gloves
- Gripper needle

Medicines:

- Medicine to take before treatment (if your doctor says you need it)
- Emergency medicine (check your Logbook for details)

Remember: Everything will be provided for you. Don't worry about getting these items yourself.

4.2 Getting Ready

NOTE: The instructions for use (reconstitution, dilution and administration) can be found in the Package Leaflet (available online at: www.medicines.org.uk/emc). A detailed description is provided in this section.

1. Prepare a clean work area and lay out the supplies.
2. The vials of Fabrazyme (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 Fabrazyme (agalsidase beta) after the labelled expiry date).
4. Count your bottles – make sure you have the right number.
5. Prepare only the number of bottles required for one infusion.



4.2 STEP 1: Preparation of the materials

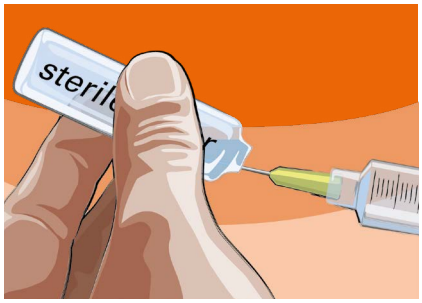
Note: The storage instructions are described in the instructions for use in the Package Leaflet (a copy of the package leaflet can be found online at: www.medicines.org.uk/emc), and must be followed.

4.3 Mixing Your Medicine

1. Remove the flip-off cap from the medicine bottle.
2. Disinfect the rubber stopper of the Fabrazyme (agalsidase beta) bottle 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 bottles, use 7.2 ml of sterile water.
   - For 5 mg bottles, use 1.1 ml of sterile water.
5. Add the sterile water slowly and gently
   - Don't squirt the sterile water in fast - this makes bubbles
   - Let the sterile water drip slowly down the inside of the bottle
   - Roll and tilt the bottle gently to mix
   - Don't shake, swirl, or turn the bottle upside down
6. If you need more bottles, repeat these steps
7. Small bubbles may appear after the mixing.
8. Wait a few minutes for the bubbles to go away and the powder to mix.
9. Check the liquid before you use it:
   - It should be clear and have no colour
   - There shouldn't be anything floating in it



4.3 STEP 2: Disinfect the vial



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

- Sometimes you might see very thin, see-through threads - this is normal

10. ⚠ **Stop and get help from your nurse and/or doctor if:**
    - You see bits floating in the liquid
    - The liquid has changed colour
11. Any unused product or waste material must be disposed of in accordance with local requirements.

4.4 Adding Your Medicine to the Salt Water Bag (Dilution)

1. Clean the opening of 1 or 2 salt water bags (NaCl 0.9%) with antiseptic solution (chlorhexidine) and let it dry.
2. Check your Logbook for how much mixed medicine you need.
3. Remove some salt water from the bag:
    - Put the needle into the bag opening
    - Slowly take out the same amount of salt water as the medicine you are going to add

    Example: If you have 14 ml of mixed medicine, take out 14 ml of salt water

    ⚠ Important: Never take out more than half the salt water from the bag
4. Remove the air from the bag:
    - Use a 50 ml syringe to pull out any air inside the bag
5. Draw up your mixed medicine
    - Slowly take the mixed medicine out of each bottle
    - Use the total amount you need
    - Make sure there is no foam in the medicine
6. Add your medicine to the salt water bag
    - Gently push all the mixed medicine into the salt water bag
7. Mix it gently
    - Turn the bag upside down slowly a few times, OR
    - Gently squeeze and massage the bag
    - Don't shake the bag or mix it too hard
8. Use the filter
    - When you give the medicine, it must go through the special filter
    - This filter is part of your tubing

4.5 Giving Your Medicine (Administration)

4.5.1 Filling the Infusion Line

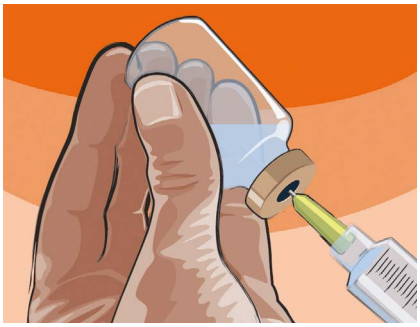
1. Get the tubing (infusion line) ready
    - Take the tubing out of the package
    - Close it using the roller clamp (the little wheel that controls the flow)
    - Attach the filter to the tubing
2. Fill the tubing with plain salt water first
    - Connect the tubing to the salt water bag that does NOT have your medicine in it



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



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

- Hold the drip chamber (the clear part) upside down
- Open the clamp to let the salt water flow in

3. Get rid of air bubbles
   - Let the salt water fill the whole tube
   - If you see any air bubbles, get them out
   - Close the roller clamp when done
4. Connect your medicine bag
   - Attach the bag with your medicine in it to the Y-shaped part of the tubing
   - Keep the clamp closed for now

4.5.2 Inserting the Needle in the Vein

⚠️ **Important: If you're doing this yourself, the adult with you must have been trained by your nurse or doctor on how to put the needle in safely.**

1. Get everything ready
   - Have strips of tape ready to use
   - Put the tubing where you can reach it easily
   - Put the antiseptic solution (chlorhexidine) and some gauzes nearby
2. Remove the needle from the packaging.
3. Get comfortable
   - Sit down
   - Rest one arm on the table (put a clean cloth down first if you can)
4. Find your vein
   - Put the tourniquet (band) on your arm
   - Look for a good vein
   - Clean the area with antiseptic solution (chlorhexidine)
   - Let it dry
5. Put the needle in
   - Pull your skin tight
   - Hold the needle with the opening facing up
   - Push it through your skin at a slight angle into the vein
   - You will see a small amount of blood appear in the tubing when the needle is in the vein – this is called a "flash"
6. Secure the needle in
   - Push the needle in about 0.5 cm (half a centimetre) so it stays in place
   - Use tape to hold it in position
   - Connect the tubing with the filter to the needle
7. Check it is working
   - Take off the tourniquet
   - The tube should fill with a little blood
   - If it doesn't, the needle isn't in the right place. You will need to try again with a new needle
   - Open the clamp for the plain salt water
8. Start your treatment
   - Set the speed according to what is written in your Logbook
   - Open the valve

- Sit back and relax during your treatment
- Keep your Logbook nearby in case you need the emergency information

4.5.3 During and After Your Treatment

When to use your medicine:

- It's best to use your medicine straight away after mixing it
- If you can't use it right away, you can keep it for up to 24 hours if you:
  - Store it in your fridge (between 2°C and 8°C)
  - Keep it away from light

⚠ Important rules:

- Your doctor decides how much medicine you get and how fast in goes
- Never change your dose or speed at home unless your doctor tells you to
- All changes must come from your doctor

When your treatment is finished:

1. Flush the tubing
   - Run plain salt water through the tubing at the same time speed as your treatment
   - This makes sure you get all your medicine
2. Remove the needle
   - Once the flushing is done, take the needle out carefully

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

What is a central venous access device? Some people have a special device fitted to make it easier to give medicine into their veins.

Learning to care for it: If you have one of these devices, your nurse will show you and your helper how to look after it properly. They might have already shown you this at the hospital.

Why is care important? You need to keep your device:

- Clear (so it doesn't get blocked)
- Clean (so it doesn't get infected)

How to care for your device:
Covering it:

- When you're using it for treatment, cover it with a see-through dressing
- When you're not using it, you don't need a dressing

Cleaning it (flushing):

- Before each treatment: Flush with 5 ml of salt water solution (NaCl 0.9%)

After each treatment:

- Flush with 5 ml of salt water solution (NaCl 0.9%)
- Then flush with 5 ml of heparin (a medicine that stops clotting)