

Global FAQs (Frequently Asked Questions)

Question	Answer
How many infusions am I likely to miss?	Currently we estimate that adults should miss one infusion in January 2009 and one in each month thereafter until Myozyme inventories are restored. The situation will be continually assessed but we are hopeful that European approval of Myozyme from the new production facility will mean that the period of tight supply will end within a few months
Will children miss infusions?	So long as the adult population cooperate with the MSWG guidelines, supply to infants and children (aged 18 years and under) should not be interrupted.
What is MSWG?	The Myozyme Stakeholders Working Group is comprised of members of the IPA, Genzyme representatives, and several physician experts in the management of Pompe disease from the US and Europe. The MSWG has met frequently over the course of several months to discuss options for clinical management of patients who could be affected by a period of intermittent delay in supply. The goal of the MSWG was to develop practical guidance for clinicians and patients to ensure that the most vulnerable patients are protected from tight supply.
What if I receive more than the standard dose of 20mg/kg every 2 weeks?	The guidance document states: "As per the Myozyme prescribing information, the recommended dose is 20mg/kg per infusion. However, if an individual has been prescribed a different dose, then it would be the treating physician's decision to maintain or change the dose."
Should every adult in the world miss an infusion?	The tight supply applies to every country in the world and affects all commercial and compassionate programs, including ICAP and MTAP in the USA. In Europe guidance to physicians is supplied by the regulatory authority (EMA) but the same action is required to protect infants and children.
My physician is on holiday at the moment, how can I arrange to miss an infusion?	If you are not able to discuss the rearrangement of your infusion dates with your hospital team, then you should contact your local patient group for advice. The IPA keeps a list of all such groups on this website at http://worldpompe.org/index.php/affiliates . Your local patient representative should be able to give you a contact at the local Genzyme office who should be able to help. Failing that please contact the IPA at one of the locations given on their website www.worldpompe.org.uk .
I had to fight for prescription/reimbursement of therapy. I'm afraid that missing infusions voluntarily will weaken my argument with physicians/insurance. What do you advise me?	The recommendation to miss an infusion is based only on the tight supply of the drug; it would certainly not be recommended for medical reasons. A similar situation has arisen in the US where patients are still waiting to start treatment, but once the supply issue is resolved we do not anticipate problems with reimbursement there.
I am a severely affected Pompe patient fully dependent on a wheelchair and on full time ventilation. Is it also recommended in severely affected late onset patients to miss an infusion?	The MSWG carefully considered all groups of patients and it was felt that there may be a very small number of severely affected patients who may need additional protection. In that case the treating physician is advised to contact Genzyme Medical Information.
Due to some organisational problems with my infusion I had to miss several infusions already the past months. Do I now again need to miss two infusions?	We would advise your physician to contact Genzyme Medical Information to explain your situation.
I just started my treatment with Myozyme. Is it then too recommended to miss two infusions?	Yes, the guidelines cover all patients aged 19 years and older.
I am a severely affected late onset patient and after a fight of several years I finally was able to start my treatment recently. Is it then recommended and medical safe (in spite of my serious and still unstable condition) to miss two infusions?	The MSWG carefully considered all groups of patients and it was felt that there may be a very small number of severely affected patients who may need additional protection. In that case the treating physician is advised to contact Genzyme Medical Information.
What will happen to me if I miss two infusions? Do Genzyme or physicians know what can happen to me?	The progression of Pompe disease is usually very slow in the adult population but it is variable from one person to the next. For this reason we would recommend that your physician monitors your situation and contacts Genzyme Medical Information if they are concerned that you have suffered a deterioration due to a missed infusion.
Do physicians know if there is a difference in impact of missing an infusion for severely affected late and less severely affected late onset patients?	The MSWG carefully considered all groups of patients and it was felt that there may be a very small number of severely affected patients who may need additional protection. In that case the treating physician is advised to contact Genzyme Medical Information.

What is the procedure when a patient is on 40g/kg/infusion Do they miss completely or are they reduced to 20g/kg/infusion for a treatment?	The guidance document states: " As per the Myozyme prescribing information, the recommended dose is 20 mg/kg per infusion. However, if an individual has been prescribed a different dose, then it would be the treating physician's decision to maintain or change the dose".
Why is it so important that I skip a treatment each month?	By anticipating tight supply we hope to guarantee that children and the most vulnerable patients will get treatment. Also we can prevent a situation where there will be no treatment available for patients whose treatment and trip to the hospital was planned.
What if the demand is increased by approvals in countries, such as India and China. How is this factored into the production requirements?	This has been taken into account. In the future this will not be a limitation. Henri Termeer mentioned in his presentation that Genzyme is estimating 5000 to 7000 patients worldwide.
Why is this tight supply not foreseen?	In each country with market approval the diagnosis of Pompe disease is not easy to predict. The increase in demand has surpassed all expectations. The increased demand had been anticipated in 2005 and production has already started in Geel. If approved there, there will be adequate stock.
Has it been considered to reduce the dose (rather than to skip)?	There have been a number of studies and there is now a strong indication that if you reduce the dose the enzyme does not reach the muscle cells. You are much better to change the dosing period and skip once, rather than to give a half dose.
How can I contact Genzyme Medical Information	Your physician will have details of the regional Genzyme Medical Information point of contact. It would be appropriate for your physician to contact them on your behalf.