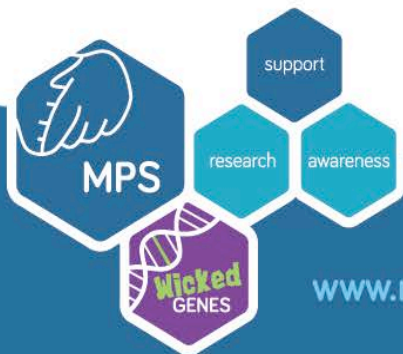


MPS Society's Role in the Shortages of Fabrazyme and Cerezyme

The Society for Mucopolysaccharide Diseases

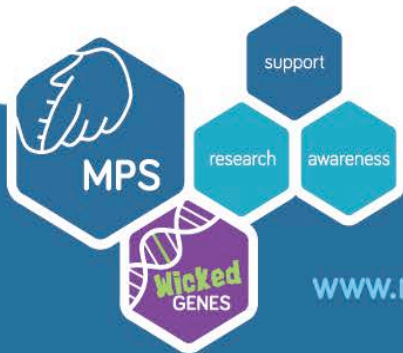
MPS House
Repton Place
White Lion Road
Amersham
HP7 9LP
Tel: 0845 3899901



Compiled and presented by Christine Lavery MBE

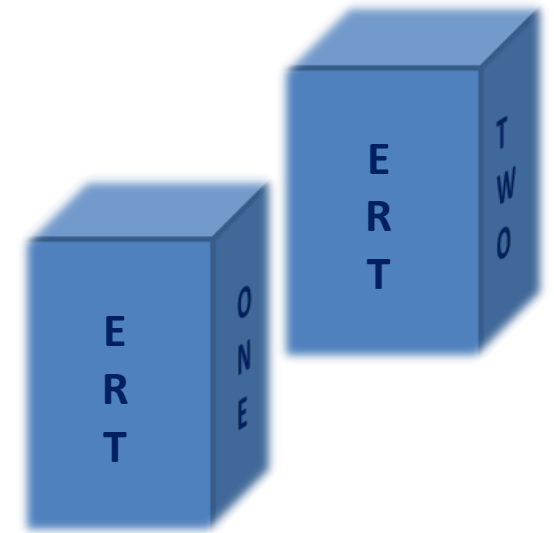
www.mpsociety.org.uk

Managing Drug Supply

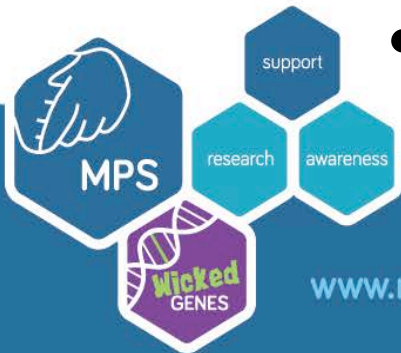


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Managing the Shortage for Patients



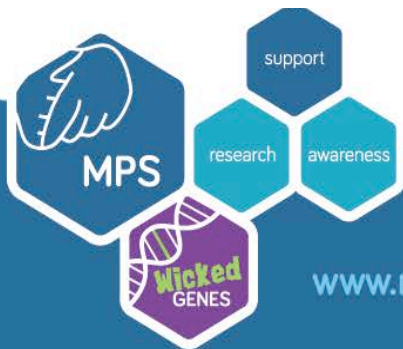
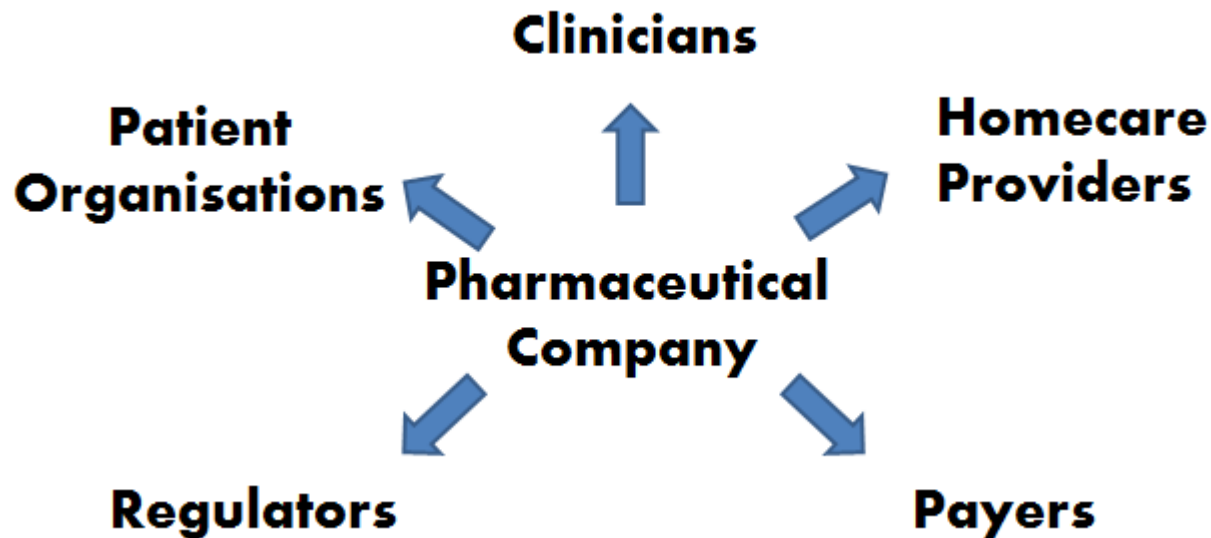
- Dose Reduction
- Transfer to another Product



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If the worst happens

- Managing Communications



Communicating on behalf of the Fabry Community

The purpose of each meeting was to receive expert opinion from regulators, payers, clinicians, Genzyme, Shire and patient representatives on

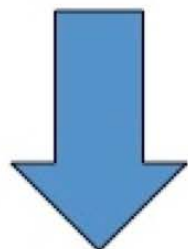
- To understand the current situation of ERT for Fabry disease
- the estimated period of time that a further shortage of ERT is expected (the critical period) based on data provided by Genzyme and Shire.
- temporary treatment recommendations on prioritization of patients to receive ERT during shortage
- follow-up recommendations of patients during and after the critical period



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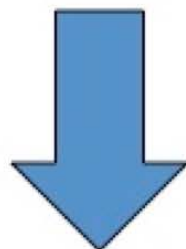
Criteria for the allocation of Replagal or Fabrazyme - Adults -

High priority



- Age of onset of disease < 50 years*)
- LVH without MRI evidence of extensive fibrosis
- GFR between 30 and 90ml/min
- Urine protein 0.3 – 1.0 gr/24 hours
- Severe acroparesthesia (if persistent after optimization of pain management)
- Cerebrovascular disease

Intermediate priority



- Less reversible disease
 - LVH with fibrosis
 - GFR < 30 ml/min
- Less severe disease:
 - Age of onset of disease > 50 years*).

Low priority



- End stage cardiac disease
- End stage CNS disease
- Multiple organ failure
- Life expectancy of less than 1 year due to other comorbidities.

Those patients mildly affected
eg, without LVH,
acroparesthesias well
controlled with optimal pain
medication, eGFR > 90.

*) Age of onset is the perceived age at which symptoms occurred which can be attributed to Fabry disease.

UK Fabry Member's Survey

At the time of the Survey

88 Fabry patients responded from 12 specialist centre

21 were on Fabrazyme; 46 were on Replagal; two on the AT1001 clinical trial and 19 were not receiving treatment

About the 39 who were on Fabrazyme at the start of the Shortage

8 continued on normal dose of fabrazyme

12 went onto a reduced dose of Fabrazyme

1 Stopped Fabrazyme and did not go onto Replagal

18 switched to Replagal from Fabrazyme between June 2009 and March 2010

How did you find out about the Fabrazyme Shortage?

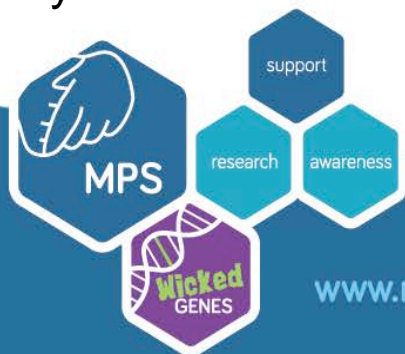
18 by the MPS Society

10 were told by their Fabry Specialist;

9 by the nurse at their Fabry Specialist Centre

5 by their ERT Homecare Company

A majority received the communication by post



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Impact of Drug Shortage for Patients

In the Survey Patients

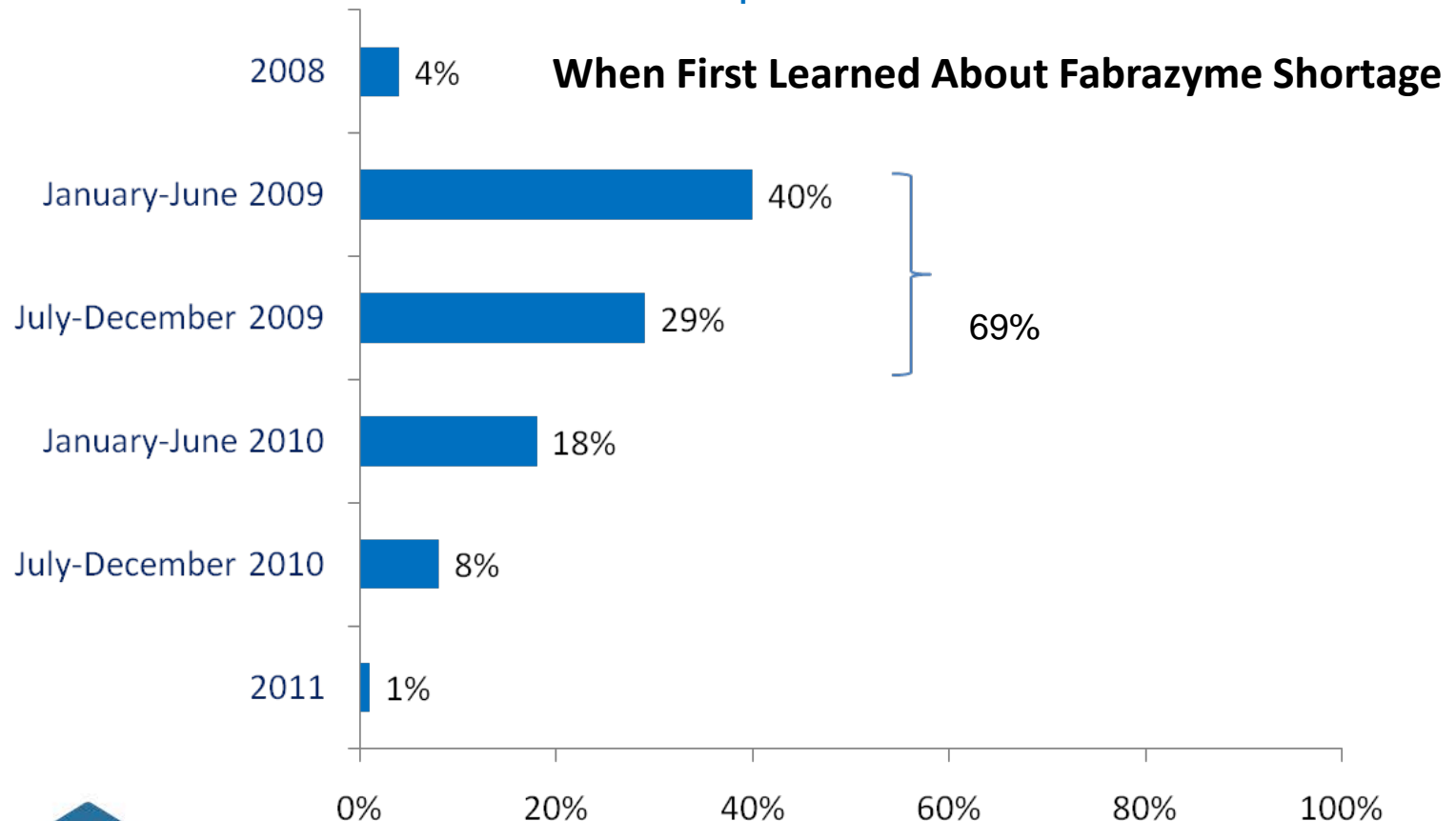
Reported the following impacts

- Uncertainty
- Anxiety
- Disease Progression
- Infusion Reactions



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Most learned about the Fabrazyme® shortage in 2009 many in June when production problems first emerged. Germany Belgium/Netherlands and the US are among the earliest to learn of problems.



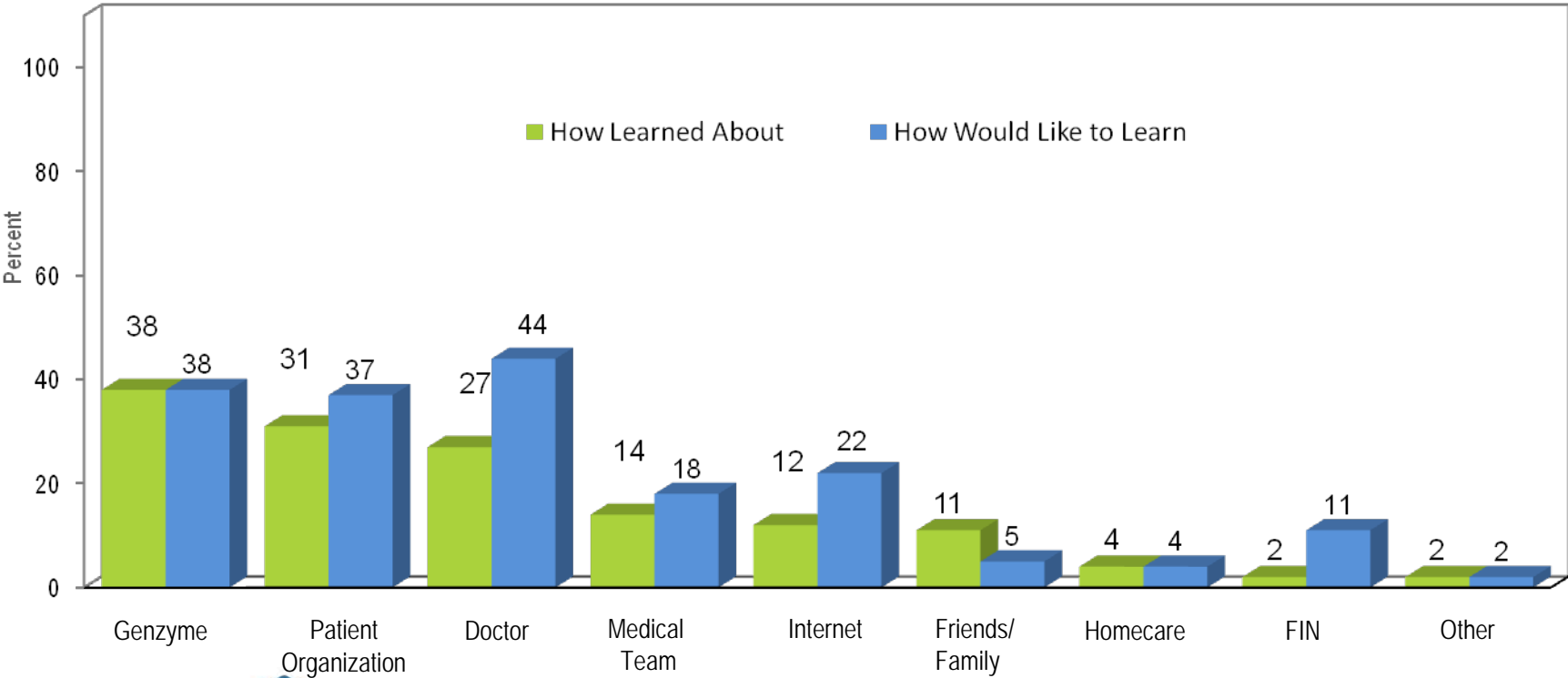
Base: Total responding/past or current users of Fabrazyme®?
Q: When did you first hear about the disruption to your supply of Fabrazyme®?



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Patients have learned about the shortage primarily through Genzyme, patient organisations and doctors. They would prefer to receive updates from the same sources, but doctors and patient organisations more.

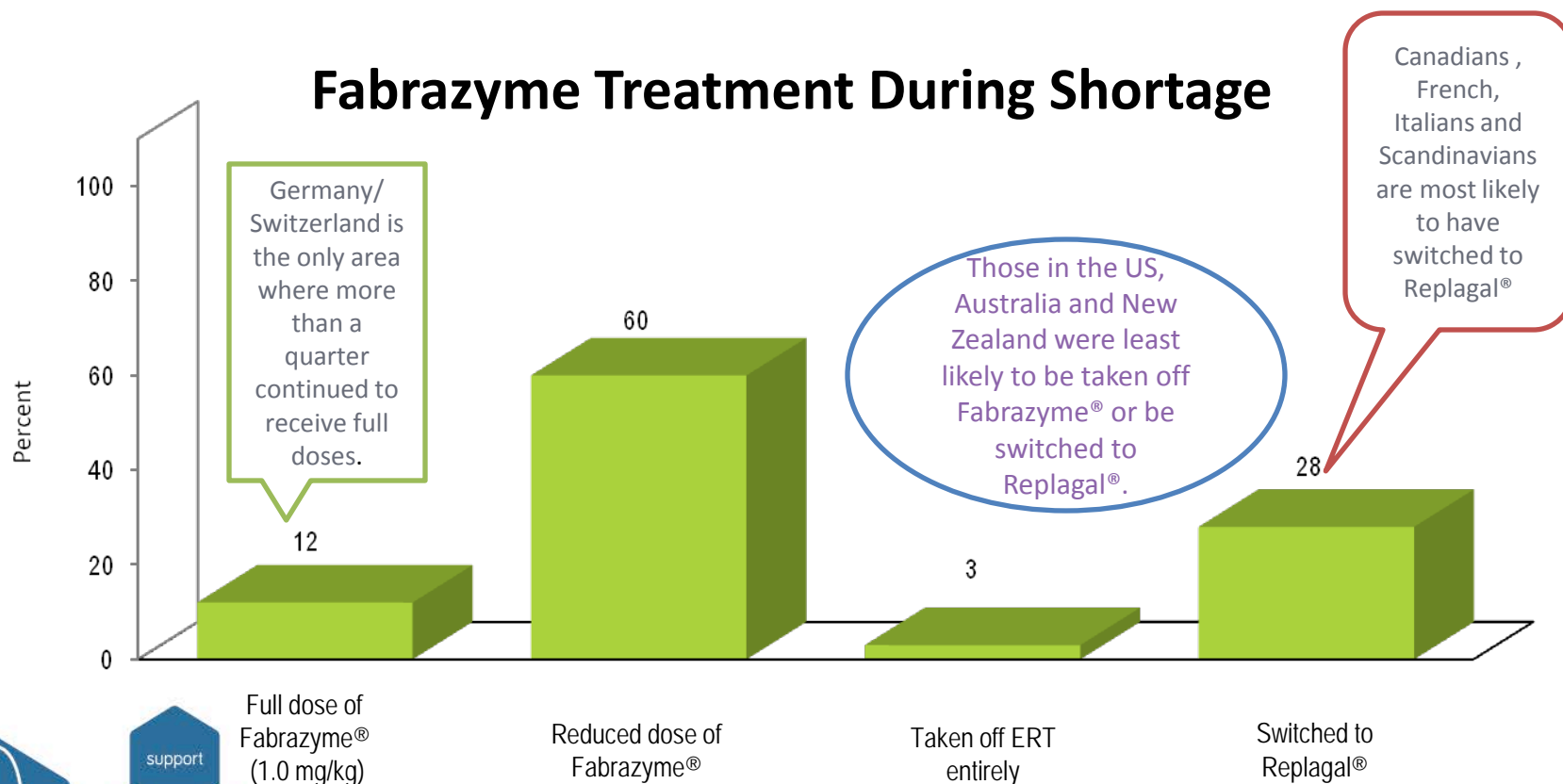
Sources of Information About the Supply Shortage



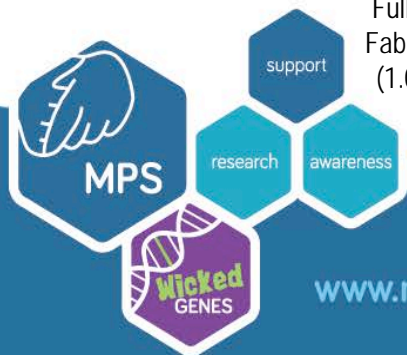
Base: Total answering (N=(347/358))
Q.: Who told you about the supply problem? Responses may add to more than 100% since some gave multiple responses.
Q.: Through whom would you like to receive your updates? Responses may add to more than 100% since some gave multiple responses.



Most on Fabrazyme® before the shortage received a reduced dose during the shortage. About a third were taken off Fabrazyme®. Few got full doses.



Base: Respondents answering/those on Fabrazyme® at time of shortage (N=234)
Q During the ERT shortage which dose were you put on?



Lessons to be Taken Forward

- Rigorous Risk Management
- Transparency of Scale of Problem
- Integrity of Communication
- Up to Date Clinical Guidelines
- Excellent Data Management



The Orphan Drug Act and Vulnerable Patients

Orphan drug legislation has really opened the door to treatment for rare diseases. However in the case of the Fabry drug shortage had there not been an alternative treatment option the situation would have been clinically devastating to patients around the world.



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