FIN Fabry Treatments Survey Report

Results of an on-line survey of the global community of Fabry Disease affected following disruption in the supply of their Enzyme Replacement Therapy, Fabrazyme®

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Acknowledgements: FIN MAB (Medical Advisory Board), FIN Industry Partners (Shire, Genzyme and Amicus Therapeutics), EURORDIS, FIN Member Country Fabry Patient Organisation Leaders, Fabry patients and their families.
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Overview
The Fabry International Network (FIN) and the Medical Advisory Board (MAB) of FIN collaborated to develop a FIN Fabry Treatments Survey about the current global Enzyme Replacement Therapy (ERT) situation. This includes the reduced supply of Fabrazyme beginning in mid 2009 and more recently the limited access to Replagal. This survey is intended to utilize the patient perspective to examine the global impact on Fabry patients resulting from these changes in ERT availability.

FIN’s vision is of a world where every person affected by Fabry disease has the best quality of life possible through early diagnosis, treatment and cure. The mission of FIN is to be a global, independent network of Fabry patient associations whose purpose is to collaborate, communicate and promote best practice to support those affected by Fabry disease.

The survey was open to all Fabry affected (male, female and children) on treatment or not on treatment. Participants were asked to complete the following survey to the best of their ability in an effort to help FIN share the important information collected with all Fabry patient organisations and stakeholders worldwide. FIN ensured all participants that no personal or confidential information will be shared.

This survey was developed by a sub-committee of the FIN Board. The Survey Committee engaged various stakeholders including; the FIN MAB (Medical Advisory Board), FIN industry partners and FIN member Fabry patient organisations. FIN would like to formally acknowledge and thank Eurordis for assisting with language translation of the FIN Fabry Treatment Survey into multiple languages. FIN would also like to acknowledge Gail Jackman of Reach Research who completed the data analysis and power point presentation for FIN and volunteers for their contributions.

Background
Production of Fabrazyme®, a major Enzyme Replacement Therapy (ERT) for those with Fabry Disease, was halted in June 2009 after a viral contamination was discovered in the sole production facility for this drug. The production interruption was expected to be temporary, but was extended several times when new issues arose. The supply of Fabrazyme® at completion date of this report remains at reduced supply globally. The length and severity of the supply interruption has resulted in most that had been on Fabrazyme® in 2009 to experience reduced access which resulted in:

- Reduced dosage and / or missed treatments
- Switching to Replagal®, another enzyme replacement therapy, or another treatment
- A patient’s involuntary vacation from treatment
Objectives

Fabry International Network, the major umbrella organisation for patient organisations serving the Fabry community is concerned with the following:

- How the Fabrazyme supply disruption has impacted those with Fabry
- What can be done now to assist those in need
- How the situation could have been / could be handled better

A survey was conducted with members of 24 Fabry organisations across 22 countries to collect the following information:

- How treatment has been impacted by the supply disruption
- Communication channels and vehicles used to learn about the Fabrazyme shortage and preferred channels and vehicles for learning about supply updates
- Ways that changes in treatment resulting from the supply interruption have impacted physical and mental health and need for medical and support services

FIN in conjunction with participating Fabry organisations, will use the information from this survey to:

- Identify the types of information and services that the Fabry community needs to cope with negative impacts from the supply shortage
- Facilitate the delivery of information and services using preferred channels and vehicles
- FIN is open to the opportunity to work with governments and industry to help develop plans to resolve ongoing issues and to handle them better if they occur.
Methodology

The FIN Survey Committee designed an online survey in consultation with the FIN Board, FIN MAB (Medical Advisory Board), FIN Industry Partners; Genzyme, Shire and Amicus as well as professional research companies. The final draft was uploaded into an online survey format. The English version was translated into 6 different languages; French, Italian, German, Polish, Portuguese and Spanish (Eurordis donated this service via their translators). The translated surveys were entered into the online survey format (totaling 7 different online surveys). The survey was sent to FIN members of 24 Fabry organisations across 22 countries. Members of the participating Fabry organisations were sent an email invitation with a link to the online survey asking them to participate. The invitations were translated from English into several languages with assistance from Eurordis translators and FIN Patient Organisation Leaders. Reminder emails were sent to most organisation members after about 1 month. The invitations and survey clearly identified FIN as the sponsor of the survey and guaranteed confidentiality of respondents’ personal information. The survey went live in December 2010 and data in this report represent all responses collected by January 31, 2011. The committee translated the open-ended responses again with assistance from FIN member patient organisation leaders.

After approaching several research companies, the committee chose Reach Research to assist with the following:

- Downloading responses from online surveys
- Code open-ended responses from English translations
- Clean survey responses
- Tabulate findings
- Write a report / summary (in power point) summarizing findings

A copy of the English version of the survey appears separately.

- A total of 442 individuals responded to the survey in time for inclusion in this report
- Data are reliable within +/- 5 percentage points at 95% confidence level for the total sample of 442
Respondent Profile

Locality
Most respondents are from North America and Western Europe. There was an opportunity for respondents to specify their country/region. The following other regions included: Turkey, Egypt, China, Serbia, and Estonia.

Age
The average age of a respondent is 45 years old.

Gender
The sample splits equally by gender.

<table>
<thead>
<tr>
<th>Region</th>
<th>Total (N=442)</th>
</tr>
</thead>
<tbody>
<tr>
<td>North America</td>
<td>52%</td>
</tr>
<tr>
<td>Europe</td>
<td>39%</td>
</tr>
<tr>
<td>Other</td>
<td>9%</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Age</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Under 18</td>
<td>3%</td>
</tr>
<tr>
<td>18 to 25</td>
<td>7%</td>
</tr>
<tr>
<td>26 to 35</td>
<td>13%</td>
</tr>
<tr>
<td>36 to 45</td>
<td>24%</td>
</tr>
<tr>
<td>46 to 55</td>
<td>30%</td>
</tr>
<tr>
<td>56 to 65</td>
<td>17%</td>
</tr>
<tr>
<td>Over 65</td>
<td>6%</td>
</tr>
<tr>
<td>Mean Age</td>
<td>45 years</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Gender</th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>Male</td>
<td>50%</td>
</tr>
<tr>
<td>Female</td>
<td>50%</td>
</tr>
</tbody>
</table>

Base: Total respondents
Q. 1: What country/region do you live in?
Q. 2: Gender
Q. 3: You’re Age
<table>
<thead>
<tr>
<th>Country</th>
<th>Number</th>
<th>Percent of Total Respondents</th>
</tr>
</thead>
<tbody>
<tr>
<td>United States</td>
<td>162</td>
<td>37%</td>
</tr>
<tr>
<td>Canada</td>
<td>62</td>
<td>14%</td>
</tr>
<tr>
<td>Australia</td>
<td>29</td>
<td>7%</td>
</tr>
<tr>
<td>The Netherlands</td>
<td>29</td>
<td>7%</td>
</tr>
<tr>
<td>Italy</td>
<td>28</td>
<td>6%</td>
</tr>
<tr>
<td>United Kingdom</td>
<td>24</td>
<td>6%</td>
</tr>
<tr>
<td>Switzerland</td>
<td>18</td>
<td>4%</td>
</tr>
<tr>
<td>France</td>
<td>17</td>
<td>4%</td>
</tr>
<tr>
<td>Poland</td>
<td>13</td>
<td>3%</td>
</tr>
<tr>
<td>Finland</td>
<td>10</td>
<td>2%</td>
</tr>
<tr>
<td>Norway</td>
<td>8</td>
<td>2%</td>
</tr>
<tr>
<td>Belgium</td>
<td>7</td>
<td>2%</td>
</tr>
<tr>
<td>Germany</td>
<td>7</td>
<td>2%</td>
</tr>
<tr>
<td>Sweden</td>
<td>5</td>
<td>1%</td>
</tr>
<tr>
<td>Brazil</td>
<td>3</td>
<td>1%</td>
</tr>
<tr>
<td>New Zealand</td>
<td>2</td>
<td>1%</td>
</tr>
<tr>
<td>Lithuania</td>
<td>1</td>
<td>-</td>
</tr>
<tr>
<td>Ireland</td>
<td>1</td>
<td>-</td>
</tr>
<tr>
<td>Denmark</td>
<td>1</td>
<td>-</td>
</tr>
<tr>
<td>Spain</td>
<td>1</td>
<td>-</td>
</tr>
<tr>
<td>Other</td>
<td>7</td>
<td>2%</td>
</tr>
</tbody>
</table>

**Countries**

- USA
- Canada
- Australia
- Netherlands
- Italy
- UK
- Switzerland
- France
- Poland
- Finland
- Norway
Limitations

Participating Fabry organisations sent email invitations to their membership requesting them to click on a link and complete the survey. Participation in the survey is likely to vary by country based on:

- Completeness and accuracy of each organization’s email lists
- The accessibility to computers and a high-speed Internet connection for email delivery and survey download. Some places there are restrictions on the use of e-mail when communicating with patients.
- Comfort with providing personal and medical information over the Internet
- Ease of reading and responding in one of the 7 languages available
- Dates when the original invitations and reminders were sent and likelihood that members would be available to receive and respond to the request

The results presented are dominated by responses from the US and Western Europe. Given that response patterns differ greatly by country/region, results should not be considered representative of global experiences, and individual patient organizations will likely want to examine the results for their country separately.

Members of patient organisations are typically the most informed and involved in disease management. Further, survey respondents tend to be ones with strong feelings or experiences that they want to share with others. Thus, while the results reported should be useful for patient organisation decision-making, they may not be typical of the larger population with Fabry Disease.

Detailed Findings

The detailed findings are categorised into the following areas:

- Health and Treatment Background
- Information about the Shortage
- Impact of Fabrazyme® Shortage on Treatments
- Impact of Shortage on Health and Well-Being.
Health and Treatment Background

Degree to which respondents are affected by Fabry disease

Over half of respondents say they are severely or significantly affected by Fabry disease. Few are not affected. Males are more affected by Fabry than females and those on ERT treatments are more affected than those on other or no treatment.

Degree to Which Respondents Are Affected by Fabry

Base: Total answering (N=427)
Q. 4: How would you say you are affected by Fabry disease?
Most respondents are on ERT and are about equally likely to be on Replagal® and Fabrazyme®. Respondents on ERT have been on treatment for mean of 6 years, regardless of which drug they currently receive. Of total respondents not on treatment: 30% do not want or need it, 15% do not qualify, 10% are on a trial, 8% cannot obtain coverage or 37% have other reasons.

**Type of Fabry Treatment at Time of Survey**

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Replagal</td>
<td>39%</td>
</tr>
<tr>
<td>Fabrazyme</td>
<td>37%</td>
</tr>
<tr>
<td>Not on treatment</td>
<td>21%</td>
</tr>
<tr>
<td>Other</td>
<td>3%</td>
</tr>
</tbody>
</table>

**Type of Fabry Treatment at Time of Survey**

- **Replagal**: 39% on treatment 6 years on average
- **Fabrazyme**: 37% on treatment 6 years on average
- **Not on treatment**: 21%
- **Other**: 3%

Those not on treatment do not want or need it (30%), do not qualify (15%), are on a trial (10%), cannot obtain coverage (8%) or have some other reason (37%).

Base: Total responding (N=425 for Q.5 and N=341 for Q.6)

Q.5: Which treatment are you currently on for your Fabry Disease?
Q.6: How long have you been on treatment?
Q.6: If you are not on treatment, why?
Where Fabry ERT (infusions) are received

Those receiving ERT are equally likely to get their ERT at home as in a hospital, generally their local hospital. Infusion location varies widely across countries. UK, Canada, France and Germany are most likely to get infusions at home. U.S, Italy, Scandinavia, Poland, Australia, New Zealand and other countries are more likely to get infusions at hospitals.
Information about the Shortage

When respondents first learned about the Fabrazyme® Shortages

Most people 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.

![Chart showing when respondents first learned about the Fabrazyme shortage]

**When First Learned About Fabrazyme Shortage**

- **Before July 2009**: 44% (73% total)
- **July-December 2009**: 29%
- **January-June 2010**: 18%
- **July-December 2010**: 8%
- **2011**: 1%

*Base: Total respondents past or current users of Fabrazyme® (N=228)  O.13: When did you first hear about the disruption to your supply of Fabrazyme®?
Sources of Information about the supply shortage

Patients have learned about the shortage primarily through Genzyme, Patient Organisations and doctors. They would prefer to receive updates from these same sources, but doctors and Patient Organisations more. Patient Organisations are most important in Italy, France and Scandinavia in terms of both how they learned and how they would like to receive updates.

**Sources of Information About the Supply Shortage**

We do not have the specific chart or graph referenced in the image to embed here. However, the chart likely shows the percentage of patients who learned about the shortage through different sources and the sources they prefer to receive updates from.

**Base:** Total answering (N=347/350)

Q.14: Who told you about the supply problem? Responses may add to more than 100% since some gave multiple responses.

Q.16: Through whom would you like to receive your updates? Responses may add to more than 100% since some gave multiple responses.
Vehicles used to communicate about the shortage

Multiple sources were used to learn about the supply disruption, with letters being the most common. Going forward, email is the most preferred vehicle for receiving updates on the situation.
Percentage believing each party is keeping them up to date (Fabry Patient Organisation, Fabry Treatment Centre/Doctor, Genzyme or Homecare Company).

A majority in all countries except for UK/Ireland believe the Fabry Patient Organisations do the best job in keeping the Fabry community informed about the Fabrazyme® shortage. At least a third in all countries except Italy believe Treatment centres and Genzyme do a good job and homecare companies do best in Canada and UK/Ireland.

Fabry patient organisations are the best source keeping the Fabry community informed about the Fabrazyme® shortage.

Percentage Believing Each Party Is Keeping Them Up-to-Date

![Bar chart showing percentages](chart.png)

**Base:** Total answering (N=approximately 800)

Q. 18: Do you feel you are being kept up to date with what is happening with Fabrazyme and its availability by each of the parties? Percentages shown are “yes” responses.
Overall, half believe more could have been done with regard to educating the Fabry community about the supply shortage. However, attitudes vary widely across countries.

**Percentage Believing More Could Have Been Done**

<table>
<thead>
<tr>
<th>Country/Region</th>
<th>Percentage</th>
</tr>
</thead>
<tbody>
<tr>
<td>Italy</td>
<td>84%</td>
</tr>
<tr>
<td>Poland/Lithuania</td>
<td>67%</td>
</tr>
<tr>
<td>France</td>
<td>60%</td>
</tr>
<tr>
<td>USA</td>
<td>53%</td>
</tr>
<tr>
<td>Germany/Switzerland</td>
<td>53%</td>
</tr>
<tr>
<td>Australia/Scandinavia/New Zealand</td>
<td>48%</td>
</tr>
<tr>
<td>UK/Ireland</td>
<td>47%</td>
</tr>
<tr>
<td>Canada</td>
<td>46%</td>
</tr>
<tr>
<td>All Others</td>
<td>25%</td>
</tr>
<tr>
<td>Belgium/Holland</td>
<td>22%</td>
</tr>
<tr>
<td>Others</td>
<td>16%</td>
</tr>
</tbody>
</table>

**Base:** Total answering (N=362)  Caution small bases on many individual country percentages.

**Q. 19:** Do you feel more could have been done? Percentages shown are “yes” responses.
Impact of Fabrazyme® Shortage on Treatments

Fabrazyme® treatment during shortage

Most on Fabrazyme® before the shortage received a reduced dose during shortage (time of survey). About a third was taken off Fabrazyme®. Germany/Switzerland is the only area where more than a quarter continued to receive full doses. At the time of the survey, Canadians, French, Italians and Scandinavians are most likely to have switched to Replagal®. Those in the US, Australia and New Zealand were least likely to be taken off Fabrazyme® or be switched to Replagal®.

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

Fabrazyme Treatment During Shortage

Base: Respondents answering those on Fabrazyme® at time of shortage (N=224)
Q. 9: During the ERT shortage which dose were you put on?
The Number of Fabrazyme® Treatments Missed Since Supply Disruption

Over two thirds have missed Fabrazyme® treatment during the shortage (at the time of the survey). About a third has missed 6 or more treatments. Europeans (except Poland) and Canadians are least likely to have missed Fabrazyme® treatments.

Over two thirds have missed Fabrazyme® treatments during the shortage. About a third have missed 6 or more treatments.

Number of Fabrazyme® Treatments Missed Since Supply Disruption

![Bar chart showing the number of treatments missed since supply disruption]

Base: Respondents answering those on Fabrazyme® at time of shortage (N=270)
Q.11: How many infusions have you missed since the Fabrazyme® supply has been disrupted?
Reasons for Stopping Treatment

The major reasons for stopping treatment are related to the Fabrazyme® shortage, and resulting missing of treatments or receiving reduced doses or switching to Replagal®.

**Respondent Comments – Appendix A (p28)**
Changes to medications since being on reduced dose of Fabrazyme® or switching treatments

Since being on reduced dose of Fabrazyme® or switching treatments 29% had made changes to other medications and 71% had not.

Changes to medications since switching to Replagal®

Since being switched to Replagal® 75% had not made changes to other medications and 25% had.

Overall a fifth of all respondents had other medications changed after adjusting for those whose Fabry treatments did not change.

Limitations
Reasons for medication changes were not requested so cause of change in medication is not established.
Who recommended change in Fabry treatment

Most changes in Fabry treatment were initiated by one’s Fabry doctor. However, individuals played an important role in many cases.

Who Recommended Change in Fabry Treatment

Base: Respondents answering those who had medications changed (N=136)
Q. 23: Who made the recommendation for you to change your Fabry treatment? Percentages may add to more than 100% because multiple responses were accepted.
Impact of Shortage on Health and Well-Being

Limitations
In interpreting the positive and negative effects, please note that the base sizes are not the same. About 140 respondents who had a change in medication said they had some sort of negative impact from the change. Only 42 of these same individuals said they had a positive impact and only 14 said they felt better or had fewer symptoms.

The negative effects since being on reduced dose of Fabrazyme®, transferring to Replagal® or stopping treatment.

21% had no negative effects, 24% had no answer and 55% reported negative effects of those whose treatment was affected. Physical symptoms, especially pain, fatigue, GI problems and neuropathy are the most common problems.

A majority of those whose treatment was affected report a negative impact. Physical symptoms, especially pain, fatigue, GI problems and neuropathy, are the most common problems.

Types of Negative Effects from Changes in Fabry Treatment (Unaided)

Base: Respondents whose Fabry treatment has changed since supply disruption (N=270)/Those with negative side effects (N=94 on Fabrazyme and 59 who switched to Replagal)
Q. 24: Since being on reduced dose of Fabrazyme, transferring to Replagal or stopping treatment, have there been any negative effects? If so, please describe.
Positive effects for those on reduced dose of Fabrazyme®

Very few reported a positive impact after changing treatments. Some who switched to Replagal® report improved health and shorter infusions.

**Respondent Comments – Appendix C (p 30)**

Participant responses are personal comments.

All treatment decisions should be made with a qualified physician.
The degree in which selected side effects occurred after change in treatment.

Consistent with the unaided responses, fatigue and pain are the side effects experienced most acutely and by most individuals.

In a separate question, nearly all (87%) said the prospect of missing or switching treatments worried them.

Limitations

- The percentages in this question are only among those reporting a problem (139 individuals out of 270 having a change in medication).

- Q26 has a variable base of 200 because not all answered. It is assumed that those respondents who did not answer did not experience the problem, but it is difficult to determine this.

- When prompted with possible problems, people are more likely to report affirmatively, even if it is an extremely minor issue on one day.

- In this instance one would assume that “A little more” means these are not problems of medical significance.

- Respondents who report some organ-related issues are not necessarily organ failure but include less favourable test results as confirmed in the range of open-ended comments received in Q2.
Frequency of monitoring of Fabry patients by Fabry specialist during the Fabrazyme® shortage.

Less than a third of those whose treatment was impacted are seeing a Fabry specialist at least quarterly. Most are being monitored only once or twice a year.

Those now receiving Replagal® are more likely to be monitored and tend to be getting tested slightly more often.

Base: Respondents answering those receiving treatment (N=245)
Q 27: During the Fabrazyme shortage, are you visiting your Fabry Specialist Center to have blood tests and monitoring?
Overall Summary
The FIN Fabry Treatments Survey has identified the following via responses:

- Many are severely / significantly affected average age being 45 years.
- Overall, males appeared more affected than females.
- Those on treatment are equally likely to be on Replagal® or Fabrazyme® and have been on treatment for mean of 6 years.
- Those not on treatment, either do not want or need it or have other reasons.
- Those receiving ERT are equally likely to get their infusions at home as those in local hospital however this varies from country to country.
- Most learned about the shortage problem in June 2009.
- Patients learned about the shortage via Genzyme, Patient Organisations and Doctors.
- Patients prefer updates from same sources but doctors and Patient Organisations more.
- Email is the most preferred vehicle for receiving updates about any changes to treatments.
- Fabry Patient Organisations do the best job in keeping the Fabry community up to date.
- Over half believe more could have been done with regard to educating Fabry community about shortage.
- Most on Fabrazyme® before shortage receive a reduced dose during shortage. A third were taken off and few got full dose.
- Two thirds missed Fabrazyme® treatments & one third missed 6 or more.
- Shortage was the main reason for stopping treatment for Fabry disease.
- About a quarter of those on Fabry treatments had changes to other medications.
- Most changes in Fabry ERT treatment were initiated by one’s Fabry Doctor. However, individuals played a role in many cases.
- Majority of those whose treatment was affected report a negative impact.
- The most common physical symptoms: pain, fatigue, GI problems and neuropathy.
- Very few reported a positive impact after changing treatments.
- Some who switched to Replagal® report improved health & shorter infusions
- Fatigue & Pain are side effects experienced most acutely by those after change in ERT treatment
- Less than a third of those whose treatment was impacted are seeing a Fabry specialist at least quarterly.
- Most are being monitored once or twice a year.
- Those now receiving Replagal® are likely to be monitored and tend to get tested slightly more often.
Conclusion

Overall the survey has revealed some interesting indications of how the supply interruption was received by the global Fabry community. The survey took advantage of a free Internet application. Participation in an online survey was not possible for everyone and this varies between global regions and dependent upon many factors including as outlined in the ‘Limitations’ page 8.

Conclusions from the FIN Fabry Treatment Survey:

- Male patients on ERT were generally more affected by Fabry than females.
- The number of females responding to the survey was equal to the number of males.
- Even though the survey was distributed in seven different languages the predominantly English speaking countries dominated the responses.
- Communication about the supply disruption reached the majority of patients in a reasonable time frame, but improvements could be made.
- Fabry patient organisations did the best job of informing the patient community about the shortage.
- Globally the majority of patients on Fabrazyme® has been on a reduced dose and has missed treatments.
- In the US and some other regions, Replagal® is not commercially available as it is not licensed and therefore unavailable as a form of treatment. The results presented are dominated by responses from the US and Western Europe. Given that response patterns differ greatly by country/region, results should not be considered representative of global experiences.
- The analysis presented is based on a collection of 442 individual responses representing a proportion of the global patient population receiving Fabry treatments, and so has value both as feedback for the FIN, Fabry Stakeholders and the Fabry population as a whole. It may also be useful for other groups facing similar difficulties in the future.
- We learnt the importance of structure and wording of surveys and how easy it is for simple questions to be misinterpreted especially when translated.

Finally FIN thanks all those who responded to the survey providing highly valued insights into personal experiences. The Fabry shortages have had a huge impact on patients and ALL Fabry stakeholders at large. Many lessons can be learned from this experience as highlighted in this survey report.

Megan Fookes
Board of Directors
Fabry International Network
Appendix A - Responses to Q7 ‘If you stopped treatment why’ Please specify

“My son and I have been cut back to once a month, I am furious; I do not want my son to suffer from this. Someone needs to do something now.”

“My Fabrazyme dose was cut in half sometime ago and I have had lots of pain because of that.”

“Since the beginning of the shortage, I have donated all of my doses of Fabrazyme to my 10 yr old son.”

“Shortage of Fabrazyme twice now switched to Replagal”

“Getting 1 time a month, not biweekly

“I am still waiting for treatment, cannot get medication for me and my children, very frustrated and feel helpless and MAD, too bad it all is because of the almighty dollar. Can someone help us????”

“We only get a treatment maybe twice in three months when we actually were getting them every other week. Because of the shortage and it has affected us.”

“Due to shortage of Fabrazyme dose was decreased by half.”

“Genzyme had problems and was unable to provide full doses of Fabrazyme.”

“I stop occasionally due to work away from home and family holidays. and after 14 years I get sick of it from time to time.”

“Price went up. And, have to pay in advance.”

“I currently receive approximately one-half of my prescribed amounts of Fabrazyme due to the shortage.”

“I am so mad about the shortage, my son, my father, and I need the meds, what in the heck is going on???”

“Nephrologists decided he did not want to continue to sponsor my IRB for Replagal. I have not had a treatment in 8 months because the closest Geneticist is 3 hours away.”

“Kidney transplantation 7 weeks”

“I initially had reduced dose of Fabrazyme, but due to increase in symptoms, I was switched to Replagal”

“Due to Fabrazyme ran out, had to change to Replagal autumn-2010.”

four times in the past year

“The only interruption was when the drug company stopped giving us the drug to get the government to pay for it.”
Appendix B – Responses to Q24 ‘Since being on reduced dose of Fabrazyme®, transferring to Replagal® or stopping treatment have there been any negative effects? Please specify’

“My Fabrazyme dose was cut in half sometime ago and I have had lots of pain”

“Incredible fatigue, constant nausea & dizziness, hearing loss, decreased kidney function, constant neuropathy, increase in angiokeratomas, increased migraines, restless legs and muscle cramps, gastro-intestinal issues and unexplained head pain”

“Severe pain in back, joints, hands, fingers. All olds problems have gotten worse. Pains returned for a while”

“I had been getting more tired when on reduced treatments of Fabrazyme. Since I started with Replagal, I have been a lot better, more energy than what I had on full treatments of Fabrazyme”

“Greater fatigue, more pain, harder to hear, balance problems,”

“Increased pains, increased diarrhoea Reduced sweating? Don’t know about any internal changes”

“Stomach problems are worse, more pain, head problems, sometimes breathing problems, lack of energy more severe than before”

“Significant increase in neuropathy in my hands and feet, increase in joint pain, decreased hearing and increased fatigue. These have resulted in missing more work and increased worry.”

“Scared to death for my Son and Father”

“I initially was on reduced dose of Fabrazyme, but due to increased symptoms, medication was changed to Replagal. Since commencing full dose of Replagal, I have been well.”

“Not that I am aware but could be something internally”

“Kidney failure”

DISCLAIMER: The included responses are individual quotes and do not necessarily represent the entire Fabry community.
Appendix C Respondents to Question 25 “Since being on reduced dose Fabrazyme, transferring to Replagal or stopping treatment, please tell us of any positive effects?”

Majority of respondents said – “None”

“Have had no difficulties”

“No longer experience chronic constipation and chronic bloating.”

“No need for EPO shots or iron supplements”

“Since transferring to Replagal, I went deer hunting by myself, go a deer, and dragged it out myself. The year before on reduced treatments of Fabrazyme, I had to have my brother-in-law drag my deer out and I could not even walk as fast as he did. On Replagal, I feel much better and can do a lot of things that I could not do before. May of 09 it took me 3 hours to mow the lawn, having to stop every 5-10 min. In September of 2010, I could do it in 45 min., like I could do before I had found out about Fabry Disease.”

“Is this a joke question?”

“Less fatigue, less pain, depression”

“Shorter infusion time”

“Found out I have a severe vitamin D deficiency”

“Since starting Replagal I am no longer in A. Fib.”

“I believe it was better when I was using Fabrazyme.”

“No reactions”

“I have been very well since being on Replagal”

“Shorter time period of infusion (one hour versus 4 hours) when switching from Fabrazyme to Replagal”

“No positive effect, other than thankful for having medicine at all”

“Since transferring to Replagal I have not noted any significant changes to my overall health.”

“I feel the same but LOVE the shorter infusion times!”

“Significantly reduced infusion time; no need for pre-meds”

DISCLAIMER: The included responses are individual quotes and do not necessarily represent the entire Fabry community.