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## Patient Relief Fund Letter



FSIG is proposing an initiative that has the potential to bring valuable benefit to those who have suffered from the Fabrazyme shortage. FIN is in support of this proposal and encourages all member organisations to give it immediate consideration. To provide your support and add your organisation's name to the letter please send an e-mail to [Jack Johnson](mailto:Jack.Johnson@fsig.org) at Include your organisation's name and that you support this letter. FIN would like to thank you for your attention to this important issue.

## European Fabry Expert Lounge Oct



Dennis Pedersen (FIN VP Europe) and Kees Bosman (FIN Treasurer) represented FIN at the European Fabry Expert Lounge this month in Budapest. The lounge allowed full group and break out discussions on critical topics related to Fabry treatments amongst the global Fabry experts. [Read Report](#)

## EMA Survey

The EMA are interested in understanding more about Patient Organisation and Consumer Organisations and their involvement with national medicine agencies. They have issued a short [survey](#) and asked FIN to distribute this to our members to complete. Responses before November 11 is appreciated.

## FDA & NORD Conferences & Workshops

Jack Johnson recently attended a series of meetings and a Drug Shortage Workshop Sep 20 through to Sep 26. Jack prepared a report giving a detailed account with some information specific to US Fabry patients and some more relevant to FIN. [Read Report](#)

The FDA has a report titled ['An Approach to Medical Product Shortages.'](#)

Jack also attended the US Conference on Rare Diseases and Orphan Products in Washington DC Oct 11-13. The conference was co-sponsored by NORD and DIA (the Drug Information Association), with collaboration from EURORDIS, FDA, NIH, and the Duke University School of Medicine. [Read Report](#)

## Genetic Alliance Awards - Fabry disease

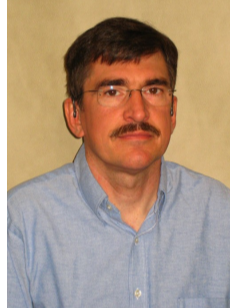


Dr. Raphael Schiffmann is a leader in his field. His research on neurogenetic disorders has led to many pivotal changes in therapy.

**Raphael Schiffmann**  
He investigated the natural history, pathogenesis and treatment of Fabry disease, an inherited disorder of lipid metabolism. Dr. Schiffmann's research concluded with the approval of enzyme replacement therapy for this disorder by association with the European Union and many additional countries. He received the Merit Award from the National Institute for his great work.

FIN MAB member; Dr Raphael Schiffmann was nominated by FIN in recognition as a leader in his field. The award was announced at the Genetic Alliance's celebration of 25 years. [Click here to view](#)

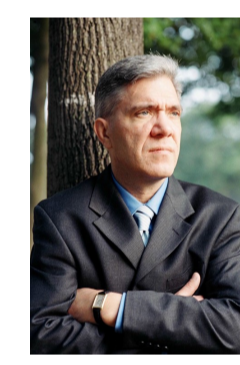
FIN Vice President (Americas); Jack Johnson was recognised for his contribution to the global Fabry family in his role as director of FSIG. [Click here to view](#).



Fabry disease is a family disease. Through FSIG, Jack has created a global Fabry family.

— John M. King  
Senior Product Director,  
Fabrazyme  
Genzyme Corporation

**Jack Johnson**  
Jack is the driving force behind Fabry Support and Information Group (FSIG). His own family's long history with Fabry inspired him to create an organization to unite people affected by Fabry to learn from and support one another. FSIG started as a "kitchen table" support group and evolved into an indispensable resource for hundreds of families.



Your leadership putting such an event together has changed our children's lives forever.

— Blanca Edna Garcia  
Fabry mother and patient

**Jerry Walter**  
Jerry founded the National Fabry Disease Foundation (NFDF) to increase assistance and support to people with Fabry disease. His advocacy work is truly innovative -- one of the best examples of his innovation is the Dallas-based Fabry Family Weekend Camp at Victory Junction, a weekend where Fabry kids and their families learn from each other and have a lot of fun!

FIN Member Jerry Walter founder of National Fabry Disease Foundation (NFDF) in recognition of the Charles Kleinschmidt Fabry Family Weekend Camp. [Click here to view](#)

## WORLD Symposium Feb 8 - 10 Meet FIN Board!



The FIN Board are attending the 8th Annual [WORLD Symposium 2012](#) in San Diego Feb 8, 9, 10. FIN will also be conducting face to face meetings with its MAB and Industry Partners. FIN would like to invite its members and Faber friends to come

and **'Meet the FIN Board Over Coffee.'** If you would like to meet with us at WORLD please contact [FIN Secretary](#). We look forward to meeting those in attendance soon.

## MAB News

FIN MAB member; Dr Gabor Linthorst mentioned the first analysis on the Fabrazyme® shortage from their group in The Netherlands, that was released. The study titled: 'Consequences of a global enzyme shortage of agalsidase beta in adult Dutch Fabry patients' documented the outcome of one year of agalsidase beta shortage and was released end of October in Orphanet Journal of Rare Diseases. [View publication](#).

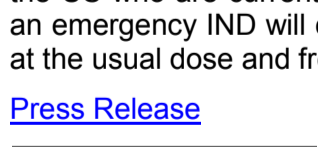
## Industry News



Following agreement with the FDA, Shire initiated the rolling submission of a Biologics License Application (BLA) for the US registration of Replagal® and will request Priority Review of this submission in response to the continuing shortage of Fabrazyme. If successful

this could mean that Replagal would be available in the US in 2012. Patients in the US who are currently accessing Replagal through the treatment protocol or an emergency IND will continue to receive uninterrupted access to their therapy at the usual dose and frequency throughout this approval process.

[Press Release](#)



Amicus announced on 20/10/2011 the close of patient recruitment for the first Phase 3 study, the FACETS Study (Study AT1001-011). This is a Phase 3 global clinical study to assess the efficacy and safety of migalastat HCl in individuals with Fabry disease who are naïve to enzyme replacement therapy (ERT). Recruitment is ongoing for the second Phase 3 study, the ATTRACT Study (AT1001-012). This is a global clinical study to measure the efficacy and safety of migalastat HCl when compared to ERT in individuals with Fabry disease who currently are receiving ERT. [Further information](#). OR [Clinical Trials](#)



Genzyme hosted the European Fabry Round Table/Expert Lounge, on October 21st -22nd in Budapest which was chaired by Dr. S Waldek. The Fabry Expert Lounge provided a platform for experts within the Fabry Disease field

to share and discuss views on the important issues facing the Fabry community as we go forward. This high level meeting had both a scientific and clinical focus; contained no basics; but provided extended opportunities for discussion. It was an open platform for communication, so as to provide an opportunity for experts to discuss the most important topics relating to Fabry disease and its treatment amongst peers.

## Contact FIN

FIN would like to hear from its membership. Please forward your comments, questions, concerns to FIN via its website. [Contact FIN](#)

