



Q2 2022 Update from IPA

International Pompe Day



Together We Are Strong
International Pompe Day

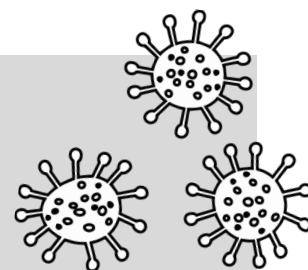
For International Pompe Day on April 15, 2022 we asked people in the global Pompe community to contribute a video illustrating how strength and resilience is gained through friendship. We received several good and informative videos.

All recordings of **International Pompe Voices** can be watched at:

www.youtube.com/playlist?list=PLZoszzG1UfTGynrfrTV0drNJRkn1yY9S4

Survey IPA/Erasmus MC Rotterdam on Covid-19

The IPA and Erasmus are working towards the issue of a new one-off survey to capture the global experience of the Coronavirus pandemic. The IPA hopes to send out a letter with instructions to patient groups soon. The Survey will be translated in several languages so as many patients as possible can participate to enhance our knowledge and to learn understand how to manage these high impact challenges on our world-wide Pompe Community in the future.



Support and Auxiliary Items



This item is to support you and your members with practical items that might be interesting. We try to be as International focussed as possible, but we also know it might not always be feasible. Please let us know when you find the same or similar items on other websites in your region of the world. We then can adapt it on our website and inform others about it.

A Seat Belt Grabber Handle to help reach your seat belt to buckle up. Simply attach the seat belt reacher around the safety belt for an instant addition of about 15cm (6 inches).

It can be bought at **Amazon.com** and look for **Seat Belt Grabber Handle**

IPA Survey to get insight and improve communication between IPA and IPA members and contacts

The IPA asked all members and contacts to fill out an online Survey to learn about wishes and comments on IPA's communication. 16 out of 54 respondents (29,6%) answered the Survey. The most important added values of an IPA membership according to respondents are: Awareness of the global Pompe Community, News on latest treatments and regular updates, Lobbying for patients interests, Collective representation with industry and researchers, Possibility to share experiences of different countries. Five members (Serbia, Japan, Mexico, South Africa, Malaysia) were interviewed via phone. A clear comment for improvement is IPA'S website that widely has been mentioned to be outdated. The IPA updates are considered to be a positive contribution to get information on research, clinical trials and other related topics. IPA will work on the outcomes of this Survey to improve accessibility of information and visibility of IPA to our members and contacts.

Motor and Respiratory decline in patients with late onset Pompe disease after cessation of enzyme replacement therapy during COVID-19 pandemic

by Céline Tard, Emmanuelle Salort-Campana, Maud Michaud, Marco Spinazzi, Aleksandra Nadaj Pakleze, Hélène Durr, Façoise Bouhour, Clair Lefeuve, Romain Thomas, Azzeddine Arrassi, Nadjib Taouagh, Guilhem Solé, Pascal Laforêt.

Eur J Neurol. 2022 Apr; 29(4):1181-1186

This article is about the impact of interruption of enzyme replacement therapy (ERT) in late onset Pompe disease. In France eight neuromuscular reference centers were obligated to stop the treatment for 31 patients. Motor and respiratory data from the French registry were collected before COVID-19 and restart of treatment. In 2,2 months, patients showed a significant deterioration of 37 meters in the 6-minute walk test and a loss of 210 ml of forced vital capacity, without restoration to original function after 3 months of ERT restarting. This study, based on the data from the French Pompe Registry shows that the interruption of ERT, even as short as a few months, worsens Pompe patients' motor and respiratory function.

Informational Document on Substrate Reduction Therapy (SRT)

SRT can be an approach in glycogen storage diseases and lysosomal storage diseases. In those diseases, a critical failure in a metabolic pathway prevents cellular breakdown and disposal of molecules. Substrate reduction therapy addresses this failure by reducing the level of the substrate (e.g. glycogen) to a point where residual degradative activity (e.g. exercise) is sufficient to prevent accumulation. It is possible that a potential SRT could be used as a monotherapy, or in combination with an Enzyme Replacement Therapy or a Gene Therapy. In the document attached to this Update you can read more information about this therapeutic strategy. This document was created by **Pompe Germany** and provided to IPA for world-wide distribution.

If you no longer wish to receive this Update, please let us know by email to info@worldpompe.org