

Chairman's report 2008

In the early part of 2008 agreements between the IPA and both the US Pompe support group AMDA and the Dutch VSN were formalised in order to provide professional assistance from Marsha Zimmerman and Paula Waddell. This was made possible through our healthy financial position and through continued support from our two main industry sponsors, Genzyme and Amicus Therapeutics. Marsha has already begun to take responsibility for the IPA publications; the Pompe connections are already requiring updates and we are considering new brochures and translations into more languages. Continued support from Paula is now assured as she acts as the IPA secretariat, organising our meetings, minute taking, and liaising with all our national affiliates.

We hope to maintain these staff positions through income from donations, grants from industry, and membership fees. We would urge all our IPA associates to help us by paying their annual IPA Membership fees when requested by the Treasurer. In 2008 we had 39 affiliated patient groups representing nearly 900 patients globally; together their small membership fees should accumulate to a very useful sum of money.

2008 also saw the launch of our new website, designed by Juan Magdaraog in the Philippines. This is a very attractive design and presents a professional image to the global Pompe community.

The IPA was relieved to see the long-awaited results from the Late Onset Treatment Study (LOTS) for Myozyme announced. The fact that Myozyme was shown to be well tolerated and that both trial endpoints were met, would hopefully weaken resistance of some nation's health providers to approval of the therapy. Progress is slowly being made towards approval in Canada, Australia, Wales, and Scotland for example – but patients are impatient to have their therapy fully funded such that access to therapy is improved and the uncertainties of continued access are permanently removed. The trial is of great importance in the USA as the FDA required this data in order to approve the 2000 litre enzyme for adults. This will be a slow process and until approval comes US adults will continue to be treated under the MTAP scheme and no new adult patients will be able to access therapy.

Industry contact and representations have continued and are set to increase over the coming years. New Enzyme Replacement Therapies (ERT) are in the pipeline from Genzyme, Amicus, Zystor, and BioMarin, and Gene therapies are under investigation by the University of Florida and Duke University. The IPA Board were invited to Geel Belgium to view progress with the new Myozyme production facility and to discuss future capacity.

In the summer of 2008 the IPA liaised closely with Genzyme as it became clear that global demand for Myozyme could soon exceed supply. We worked through weekly teleconferences with Genzyme staff and leading Pompe physicians to prepare a clear set of guidelines for physicians and patients in order to reduce the demand until Geel production could be made available. Thankfully most patients were only required to miss one infusion in early 2009 but the preparation was certainly well worthwhile and it is hoped that a 2009 feedback survey will show how well the communication plan was designed and implemented.

The IPA invited international patient groups to its AGM and patient meeting in Reading, UK. As expected very few international representatives attended the meetings, other than the IPA Board members and its advisors. However the meeting was fully reported and both PowerPoint

presentations and YouTube videos were made available through the AGSD-UK Pompe website (www.pompe.org.uk); so the meeting reached a wide global Pompe community.

The involvement of the IPA in new developments for therapies is set to place greater burdens on the Board throughout 2009, but with the end of the tight supply issue, the LOTS results and the help of our professional staff, we hope to be able to concentrate on our core objectives of supporting national groups and providing accurate information to patients.