



December 16, 2014

As many of you are probably aware, on December 13, 2014 an interview was published in Argos (a Dutch radio program) regarding a potential new production method for a treatment for Pompe disease. A short summary of the interview was made available in English on the website: [www.utrechtcentral.com](http://www.utrechtcentral.com).

The International Pompe Association (IPA) strongly supports any research into Pompe disease that will increase disease knowledge, improve current treatments (through better efficacy or better price), or lead to better disease management. We also strongly believe in providing the Pompe community with clear, unbiased information regarding developments in the field of Pompe research.

After the publication in Argos, the IPA and our member organizations have received numerous inquiries regarding the announcement in the article that “a new, more economical method” for producing enzyme replacement therapy for Pompe disease could be possible within the next two years.

The IPA fully supports any efforts that will lead to more cost-effective treatments, as well as more effective treatments in general. However, at this time it is also important to understand the context in which the statements in the article were made.

According to a follow-up interview with Professor Huub Schellekens of the University of Utrecht in the Volkskrant, his statements were regarding a proposed shift in policy in the Netherlands that he is advocating for. He believes that rather than the existing system of mass-production by industry, a more cost-effective solution would be for individual hospitals in the Netherlands to make drugs (including ones like enzyme replacement therapy for Pompe) in their own pharmacies using his proposed method.

He specifically stated: “We do not intend to bring this product on the market. I am fighting to make drugs widely available at much lower prices. We are now in a system with exploding prices for rare drugs that people cannot do without, such as Pompe disease. I want to bring the system into question.”

It is important for the international community to understand that there is an ongoing debate within the Netherlands (as in many countries around the world) about the high price of therapies for rare diseases. As part of this debate, new ways to produce and market drugs will continue to be proposed and discussed. In this particular case, the proposal is to change the current system completely. According to Professor Schellekens, there is no intention to take the proposed production method and make it a commercially-available product on an international scale.

The IPA remains committed to doing our best to keep the international Pompe community informed about all developments in Pompe disease research and will continue to monitor the developments mentioned above.

If you have any questions or would like to discuss these items more, please contact us: [info@worldpompe.org](mailto:info@worldpompe.org).

Thank you.

The IPA Board