

# 03.10.2024 IPA Update Q4/2024

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- **AMDA/IPA Scientific Conference Presentations Available Online!**  
*Die Präsentationen der AMDA/IPA International Pompe Patient & Scientific Conference vom 3. bis 5. Mai 2024 sind jetzt online verfügbar: [2024 AMDA/IPA Conference - YouTube](#)*
- **#Pies4Pompe Challenge**  
*Die #Pies4Pompe Challenge ist eine Awarenesskampagne der Canadian Association of Pompe. Sie wurde am Internationalen Pompe-Tag 2024 gestartet: [#Pies4Pompe Challenge \(youtube.com\)](#)*
- **Sharing experiences - a Pompe family in Australia**  
*Wir suchten nach Möglichkeiten, die Erfahrungen unseres Sohnes im Krankenhaus zu verbessern und stießen auf die „4 Paws On The Floor“-Tiertherapie.*
- **Scientific Articles on Pompe Disease**  
*Aktuelle wissenschaftliche Pompe-Publikationen.*



International Pompe Association

## IPA Update Q4 2024

### AMDA/IPA Scientific Conference Presentations Available Online!

The presentations of the AMDA/IPA International Pompe Patient & Scientific Conference held at May 3-5 2024 are now online.

They can be viewed at [2024 AMDA/IPA Conference - YouTube](#).



### #Pies4Pompe Challenge

The #Pies4Pompe Challenge is an awareness campaign hosted by the [Canadian Association of Pompe](#) and driven by community and public participation. It was launched on International Pompe Day 2024!

Click the link to view some pie action >> [#Pies4Pompe Challenge \(youtube.com\)](https://www.youtube.com/#Pies4Pompe)

Why? Because **Pompe disease is severely under-diagnosed in Canada**, with fewer than 70 patients. Data from newborn screening programs in other countries suggest that there could be 30 times more diagnoses. Even if this estimate is a bit high, it highlights that we are missing a significant number of patients. Adding Pompe disease to newborn screening panels in Canada would ensure that newborns aren't missed. But, what about everyone else?

What can we do? We can talk about it. We can engage our friends and their friends. Taking a pie and challenging a friend to do the same creates conversation. Conversation leads to knowledge and awareness. *And there's nothing wrong with having fun while we do it!*

Find more information about the #Pies4Pompe Challenge on [Pies4Pompe.com](https://Pies4Pompe.com).

[#Pies4Pompe Challenge - Canadian Association of Pompe \(pompecanada.com\)](https://pompecanada.com/#Pies4Pompe)



### Sharing experiences - a Pompe family in Australia

*We were looking for ways in which we could improve the experience for our son at hospital and stumbled across 4 Paws On The Floor Pet Therapy, located on the Gold Coast in Queensland. We arranged for the dog trainer to come to the hospital with Oreo, a delightful brown and white Labradoodle, and it went so well that we have now arranged for Oreo to come for every infusion day.*

*Oreo sits on the bed with our son and has cuddles, pats and treats. They have formed a special bond, with our son whispering things into his ear, and Oreo will place his paw in our sons lap when our sons blood pressure is being measured. Oreo is very calm and relaxed and the visits are great for not only our son, but for us (his parents) too, as the visit gives us all a little break as well as something to look forward to.*

*Oreo is non-shedding which is fantastic - no dog hair on the hospital bed or on anyone's clothing. He is always clean and well-groomed too. The other children in the ward also love his visits, as do the staff.*

<https://www.4pawsonthefloor.com.au/who-we-help/>



### Scientific Articles on Pompe disease

**Changes in forced vital capacity over  $\leq 13$  years among patients with late-onset Pompe disease treated with alglucosidase alfa: new modeling of real-world data from the Pompe Registry.**

Chronic respiratory insufficiency from progressive muscle weakness causes morbidity and mortality in late-onset Pompe disease (LOPD). Previous Pompe Registry (NCT00231400) analyses for  $\leq 5$  years' alglucosidase alfa treatment showed a single linear time trend of stable forced vital capacity (FVC) % predicted.

<https://clmz.nl/en/publication/38896264/changes-in-forced-vi...>

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**Start, switch and stop (triple-S) criteria for enzyme replacement therapy of late-onset Pompe disease: European Pompe Consortium recommendation update 2024.**

Two novel enzyme replacement therapies (ERTs), studied in phase 3 trials in late-onset Pompe patients, reached marketing authorization by the European Medicines Agency in 2022 and 2023. The European Pompe Consortium (EPOC) updates and extends the scope of the 2017 recommendations for starting, switching and stopping ERT.

<https://clmz.nl/en/publication/38873957/start-switch-and-sto...>

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**Long term survival in patients with classic infantile Pompe disease reveals a spectrum with progressive brain abnormalities and changes in cognitive functioning.**

This study, is to provide more insight into the pattern of brain abnormalities, and possible consequences for cognitive functioning, in patients with classic infantile Pompe disease.

<https://clmz.nl/en/publication/38584574/long-term-survival-i...>

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