



**Young Investigator
Excellence in Research Programme**
Amicus Therapeutics

Opportunity to Spotlight Your Research in Pompe Disease

**Invitation to submit an abstract to Amicus' Young Investigator
Excellence in Research Programme**

Amicus Therapeutics' Executive Chairman and founding Chief Executive Officer, John F. Crowley, has based his every decision on his mission to save the lives of his two children who live with the severe neuromuscular disorder Pompe disease. He has worked tirelessly as a parent, patient advocate, and entrepreneur to bring hope to people living with rare diseases, their caregivers, and their families worldwide. It was in his drive to find a treatment that Amicus Therapeutics was launched in 2005, to reimagine the future of rare diseases, and continue his mission.

In accordance with Amicus' founding principles and John's quest, we are excited to invite healthcare professionals who have undertaken research in Pompe disease to partake in our Young Investigator Excellence in Research Programme.

This programme, sponsored by Amicus Therapeutics, aims to recognize the latest research in Pompe disease, while facilitating the sharing of insights and information in the wider scientific community. We encourage healthcare professionals, scientists, and allied healthcare professionals to submit an abstract of their independent research, which is sufficiently complete to be shared. Together with Amicus, a panel of international experts in Pompe disease will extend the opportunity to successful applicants to present their research during a dedicated live, peer-to-peer meeting in April 2023 (travel and accommodation will be provided). Successful applicants will also be announced via the Amicus website and social media, and receive a certificate confirming their involvement in the Young Investigator Excellence in Research Programme.

Following the presentations and award ceremony, attendees will be invited to attend a networking dinner, and will also have the opportunity to attend a dedicated Pompe disease medical education event the following day. The applicant whose submission is judged to be the strongest and most impactful by the expert panel will be invited to present their research at an Amicus educational event in the subsequent year.

If you are interested in submitting your research to the Young Investigator Excellence in Research Programme, please send your abstract to Amicus via amicusyounginvestigators@comradis.biz before the deadline 6th January 2023. The submitted abstract will be shared with our panel of international experts and successful applicants will be notified by February 2023. Further information including eligibility criteria is available below.

Please note that the abstract submission process will be managed by our partner agency Comradis, who will be responsible for communicating whether applicants have been successful, on behalf of Amicus Therapeutics.

Job number: NP--IN-00041022

Date of preparation: October 2022

Applicants' Eligibility Criteria:

- Applicants should be active in the field of Pompe disease (research, clinical practice or other healthcare); prior publications in Pompe disease research are not a prerequisite
- Applications are welcome from those with prior experience in other fields who are new to Pompe disease
- This programme is encouraging burgeoning researchers in the Pompe disease field. Therefore, applicants should not already hold a fixed, senior position in the Pompe disease field
- Amicus will not provide funding for research in this programme

Suggested Research Topics (this list is not exhaustive):

- Research should be focused on late-onset Pompe disease (LOPD) or infantile-onset Pompe disease (IOPD)
- Basic and translational research (including biochemistry and pathophysiology, but excluding drug efficacy)
- Time to diagnosis
- Patient management research (e.g., care pathways, supportive therapies)
- Role of diet/nutrition/exercise
- Patient case studies
- Monitoring disease progression (including subclinical changes, quality of life, multidisciplinary team coordination, new monitoring techniques such as wearable devices)

Abstract Guidelines:

- Include: Title, author names, and institutions
- Structure: Introduction, Aims, Methods, Results, Conclusion
- All abstracts must be in English
- Maximum 2000 characters, excluding spaces, title, author names, and institutions
- One figure or table may be included (excluded in character count)
- Applicants must declare if some/all data in the abstract have been previously presented and provide details of where/when; previously published data are not accepted
- Interim analyses of ongoing work will be accepted – insofar as ready to be presented in April 2023