The Inozyme Advocate

Your source for news, information, and events on Rare Calcification Disorders

Vol. 4 | April 2020

Welcome to The Inozyme Advocate, a semi-annual communication to bring you news, information, and events from the Patient Advocacy Team at Inozyme!

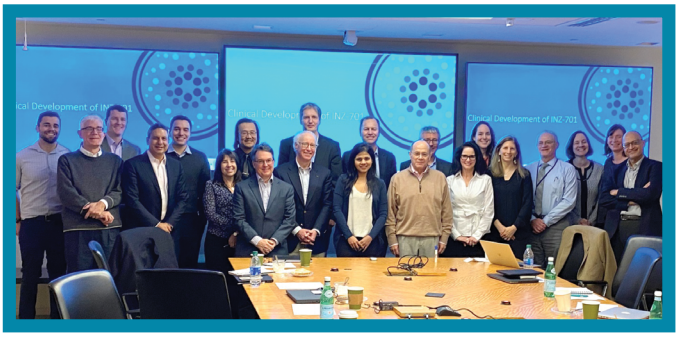
We are Making Great Progress!

Inozyme is growing

In the last few months, Inozyme has added new members to the team in key roles.

- **Elizabeth Alessi-** Regulatory Affairs
- **Damon Banks-** Vice President, Legal Affairs, Corporate Counsel
- **Montagu Hankin-** Director, Program Management
- **Jennifer Howe-** Preclinical Program Manager
- **Pedro Huertas-** Chief Medical Officer
- **Gus Khursigara-** Vice President, Medical Affairs & Clinical Operations
- **Angela Lynch-** Consultant to full time employee March 2020
- **Bridget McNamara-** Office Manager
- **Catherine Nester-** Vice President, Physician and Patient Strategies
- **Michele Rosner-** Director, Clinical Studies, Medical Affairs

3rd Inozyme Clinical Advisory Meeting- Chevy Chase, Maryland



Pictured from left- Nathan Kucera, Dr. Tom Carpenter, Montagu Hankin, Gus Khursigara, Dr. Carlos Ferreira, Beth Leiro, Qing Liu, Dr. Demetrios Braddock, Dr. Michael Levine, Henric Bjarke, Supriya Rao, Axel Bolte, Dr. Pedro Huertas, Dr. Frank Rutsch, Catherine Nester, Dr. Shira Ziegler, Dr. Rachel Gafni, Dr. Bill Gahl, Dr. Deborah Wenkert, Karen Wesley, and Dr. Zulf Mughal.

On December 7th Inozyme held the 3rd annual Clinical Advisory Meeting in Chevy Chase, Maryland. The meeting brought together many of the world's experts in ENPP1 deficiency, covering both the infantile form, general arterial calcification of infancy, or GACI, and post infancy form, autosomal recessive hypophosphatemic rickets type 2, or ARHR2. The purpose of the meeting was to review the newly completed ENPP1 deficiency Natural History Study, discuss the current understanding of the patient experience and gain consensus and agreement on the clinical plan. Other topics reviewed were the diagnostic strategy and the current thinking on the disease story. The Inozyme team was able to gain valuable input on all topics and will use this to further refine the plan for INZ-701, our investigational therapy for the treatment of patients with ENPP1 deficiency, moving forward. An expanded global advisory board is planned for later in the spring of 2020 and will be used to gain additional input from clinicians who participated in the Natural History Study globally.

Patient Research Project Kicked Off

Inozyme, in collaboration with GACI Global and Engage Health, has initiated work on a patient research project. The purpose of this research is to gain a deeper understanding in their own words of what patients with GACI and/or ARHR2 and their families experience daily. The target number of interviews for the project is 90 and will involve patients from around the world. The Co-Principal Investigators for the study will be Dr. Pedro Huertas, the Inozyme CMO and Christine O'Brien, Co-President of GACI Global.

Rare Disease Day 2020

Rare Disease Day is observed worldwide annually on last day of February to raise awareness for rare diseases and the daily impact living with a rare disease has on patients and families. This year, Inozyme celebrated this event by hosting Inozyme scientific founder, Dr. Demetrios Braddock, as well as members of the GAGI Global community. We were joined by the Molloy, Shea, and O'Brien-Lemansky families. Inozyme shared their updates on the effort towards helping these patients and their families and their goal of developing a safe and effective treatment for this severe and debilitating disease, while Dr. Braddock told of the long road he traveled to develop an enzyme replacement to treat ENPP1 deficiency, from his early work in the lab at Yale University to the current status of his research today. In addition, members from the GACI Global community GACI Global shared their experiences with ENPP1 deficiency and their journey from getting an initial diagnosis to the challenges in caring for children with a rare, life threatening disease. It was extremely motivating for Inozyme to understand what the GACI Global patients and families have been through.

GACI Global

GAGI Global has made significant progress in advancing its mission to reach more families affected by GACI and ARHR2 and has continued to raise awareness in the community.

Some of the key highlights include:

- Fall and Winter fundraisers
- Plans for a worldwide walk on May 16, 2020
- Updates to the Wikpedia page to include the most recent research and information
- GACI and ARHR2 were successfully listed on NORD's rare disease website
- Communication about GACI Global to over 200 medical professionals and researchers who have published on the disease

Key Events - 2020

The following meetings maybe of interest to patient, caregivers and HCPs. Visit their websites to find the ones of interest to you.

- **Aug 25-29, 2020: ACMG Annual Clinical Genetics Meeting,** San Antonio, Texas
  acmgmeeting.net/acmg2020/Public/mainhall.aspx
- **Oct 20-24, 2020: European Calcified Tissue Society,** Marseille, France
  ects2020.org/
- **Sept 11-15, 2020: ASBMR CME Event,** Seattle, Washington
  asbmr.org/meetings/annualmeeting.aspx
- **October 27-31, 2020: American Society of Human Genetics,** San Diego, California
  ashg.org/meetings/2020meeting/

inozyme pharma

INOZYME PHARMA
280 SUMMER STREET, FLOOR 5
BOSTON, MA 02210

PHONE: +1 (857) 330-4340
INFO@INOZYME.COM

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