

Dear Cystinosis Community,

We hope that you are enjoying the fall. The Novartis teams have been hard at work collaborating with the cystinosis community, cystinosis patient advocacy groups, cystinosis experts and health authorities to move the program forward since the Novartis acquisition of the investigational autologous hematopoietic stem cell (HSC) gene therapy program for the treatment of cystinosis from AVROBIO last year.

Specifically, this year we have been honored to sponsor and attend three patient advocacy group meetings, including the Cystinosis Research Foundation (CRF) Day of Hope in April in San Diego, the Cystinosis Research Network (CRN) Cystinosis Symposium in May in New York City, and most recently the Cystinosis Network Europe (CNE) International Conference in July in Manchester, UK. These meetings have been invaluable to our teams as we connect and learn from the community.

To further ensure our clinical program includes the voice of the patient, we have conducted three activities which have provided the opportunity for broad involvement of the community on the program. These include a planned review with the CNE Cystinosis Community Advisory Board (CAB) this Fall, and an advisory board made up of US caregivers of individuals living with cystinosis which took place earlier this summer. Both seek to learn about the lived experience of cystinosis including challenges and unmet needs, understand perspectives on the current standard of care for cystinosis, gain insights on educational needs around investigational autologous HSC transplantation and clinical trials. Insights gained from this event will be extremely helpful as our team moves the program forward.

Earlier this summer work was done to gain an understanding of the cystinosis diagnostic odyssey, burden of disease and treatment burden. Forty caregivers and individuals living with cystinosis were interviewed for the project. It became clear that it is important to recognize that the cystinosis patient experience is different today than it was in the past and that while therapies now exist to address the pressing concerns of disease progression, there remain still unmet needs to be addressed.

Novartis continues to be committed to working with our partners in the cystinosis community to hear the community voice. The cystinosis advocacy community are strong allies who we are partnering with to deepen our understanding of this disease. We are grateful for your willingness as a community to share your experiences living with cystinosis and tell us about your hopes for the future.

We look forward to continuing our close collaboration with the cystinosis community and will provide you with updates as we have more information available to share.

Sincerely,

The Novartis Cystinosis Development Team

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