Lysosomal & Rare Disorders Research & Treatment Center

www.lysosomalcenter.org

3702 Pender Drive, Suite 170 Fairfax, Virginia 22030 Tel: (703)261-6220 Fax: (703) 991-6592 Email: info@ldrtc.org

LDRTC Annual Report 2021 and 2022

We are presenting our annual report to share our main accomplishments for the year 2021 and 2022. LDRTC team worked tirelessly on numerous clinical consultations, clinical and bench to bedside research to bring our community the latest treatment options in Lysosomal Disorders.

During last year, our team conducted over 5000 patient visits, including Genetics, Clinical Research, and Infusion visits. At LDRTC, we are fully committed to giving our patients the time and attention to understand the scope of medical issues and develop management and treatment plans. We are able to spend days, if needed, with the patients and families to find the root of the issues and offer help with a '**whole-listic**" approach.

As a result of LDRTC's commitment to bring the Rare Disease patients' reasons to be hopeful for the future, we are proud to announce that we have dosed multiple patients in gene therapy clinical protocols. This disease-modifying treatment is one of the most promising therapies to treat the underlying condition and improve the quality of life of patients impacted by these debilitating diseases.

We focus on the most up-to-date research to guide our medical advances. LDRTC's primary goal is to keep bringing new understanding to the different aspects of Lysosomal diseases and unravel new treatment options for patients with rare disorders. Through bench to bedside translational research, sponsored and investigator-initiated trials, we conducted multiple clinical studies delivering new and novel therapies for rare diseases.

We are proud to have been directly involved in the research that has yielded FDA approval for therapies for rare disease patients all over the country and the world. Part of our mission is to create a greater understanding of Lysosomal Disorders not only through research but providing treatment through clinical trials and enabling the patients to try new therapeutic options prior to the FDA approval.

The projects conducted by the Translational Research Unit not only yielded significant findings on LDs but also awarded one of our trainees with the "Young Investigator" award. We have shared our knowledge on several CME Webinars, conferences, lectures, publications, and manuscripts.

The gaps in healthcare for patients with Lysosomal disorders are further augmented by the paucity of experts to train the medical community. We understand the importance of spreading our knowledge in LDs. Through our Healthcare Provider Fellowship Program in Lysosomal Disorders, we have been able to expand and share our expertise. Our program hosted 7 fellows, including the rotating Clinical Genetics fellows during the year 2021-2022.

LDRTC also hosted annual Genetic, Rare & Immune Disorders (GRIDS) symposia gathering world-renowned physicians, researchers, and patient advocates to discuss the latest findings in lysosomal disorders. Nearly 12,000 attendees have benefitted from this event via in-person, virtual or through the CME continuing education initiative.

We're looking forward to contributing to new medical advances in the upcoming years!

Thank you,

Ozlem Goker-Alpan, MD | Founder