Our Commitment to Patients

“Our ongoing commitment is to develop first-ever approved treatments for rare and ultra-rare diseases and strive for majority access globally to our medicines for patients who could benefit from them. We are also committed to helping the rare disease community move forward by sharing our science and expertise to advance treatment development, whether by us or others.”

EMIL D. KAKKIS, M.D., PH.D.
FOUNDER, CEO, AND PRESIDENT

WE ARE COMMITTED TO ADVANCING INNOVATIVE LIFE-CHANGING TREATMENTS, PROVIDING ACCESS TO THOSE IN NEED, & SUPPORTING THE LARGER RARE DISEASE COMMUNITY

ADDRESS UNMET NEED

ENSURE ACCESS

SUPPORT RARE DISEASE COMMUNITY

We believe the long-term value of **innovation** in healthcare is critical for the many patients with rare diseases who are still waiting for an approved treatment. We therefore dedicate ourselves every day to the **goal of providing treatments where none currently exist.** This commitment is based on the fundamental principles of advancing innovative life-changing treatments as quickly as possible and **effectively providing these treatments to patients in need.** We aim to develop safe and effective treatments for many serious rare diseases as fast as we can, and we are **committed to helping the whole rare disease community move forward** by sharing our science and expertise to advance future development, whether by us or others.

Innovation in Action: Matthew was diagnosed with mucopolysaccharidosis type VII, or MPS VII, when he was 17 months old and he is now 20 years old. When Matthew was a toddler, his mother approached Dr. Emil Kakkis, our President and CEO, hoping he could develop a treatment for Matthew. Ultragenyx was able to license rights to develop a treatment for MPS VII in 2010 and begin development. In 2013, Matthew’s mother urgently contacted Dr. Kakkis about treatment for Matthew because his lungs were failing and she did not believe Matthew would live much longer. Although no one with MPS VII had been treated at that point, Ultragenyx decided to treat Matthew on a compassionate use basis because it was expected that Matthew would not survive long enough to be in a clinical trial. Matthew became the first patient with MPS VII to be treated, and he is a loving child with an infectious smile who enjoys time spent with family and friends.

WE URGENTLY DEVELOP TREATMENTS WHERE NO SUFFICIENT TREATMENT OPTIONS EXIST

We recognize that **more than 90% of rare diseases do not have treatments available** and that it is of the **utmost urgency to find treatments where none currently exist.** Of our four approved medicines, three of them — Crysvita® Mepsevii® and Dojolvi® — are the only approved therapy for their respective diseases. Additionally, 17 of the 18 diseases we are targeting do not have an existing approved therapy that directly addresses the underlying disease in the majority of patients. To **develop these first-ever treatments** effectively, we also **engage patients and their caregivers** during development in a two-way conversation to understand how the diseases affect their lives and **so that they can provide us with insight to develop treatments that will be meaningful to them.** We believe it is our responsibility to utilize innovative development techniques (e.g., adaptive trial designs and seamless Phase 1/2/3 trials) to accelerate the drug development process.

CRYSVITA (burosumab-twza)

The only approved therapy for X-linked hypophosphatemia (XLH) and tumor-induced osteomalacia (TIO)

Mepsevii® (vestronidase alfa-vjbk)

The only approved therapy for mucopolysaccharidosis type VII (MPS VII)

DOJOLVI TRIHEPTANOIN Oral Liquid

The only approved therapy for long-chain fatty acid oxidation disorders (LC-FAOD)

5.5 Average number of years from entering the clinic to approval compared to ~7 to 7.5 years for our peers

10 New evaluations or tools developed to improve drug development based on patient feedback

WE STRIVE FOR PATIENTS TO HAVE ACCESS TO OUR THERAPIES AS SOON AS POSSIBLE

We believe that we have an obligation to develop the most effective medicines and **ensure global majority access for patients who can benefit**. We are committed to pricing our medicines responsibly and ensuring any price increases are consistent with inflation adjustments. In the U.S., we will ensure that no patient forgoes treatment for financial reasons, including affording copays or lack of insurance. We also provide early pre-approval access to our therapies when medically appropriate.

We would only consider increasing the prices of our medicines in a manner consistent with inflation adjustments

~25 Countries where patients have access to our therapies

~220 Patients treated via compassionate access with our medicines (Crysvita®, Mepsevii® and Dojolvi®) since 2013

WE SUPPORT THE RARE DISEASE COMMUNITY

We are **committed to helping patients obtain early and accurate diagnoses** whether or not they have a disease that we are studying, as well as **helping the field move forward by sharing our science and expertise to advance future development,** whether by us or others. For example, we share de-identified natural history data, as well as endpoints, methods and validations we have developed, with both physician groups and patient groups. We also host an annual boot camp designed for patients and advocates who have started funding rare disease research and are looking to better coordinate and build structure around their efforts. One prior boot camp attendee went on to found GeneTx Biotherapeutics to treat Angelman Syndrome, a disease with no approved therapy. We have partnered with (and recently acquired) GeneTx to develop their investigational drug, which is currently in clinical development. We will always help patients, foundations and other companies seeking to develop novel treatments for rare diseases using our knowledge, insights, experiences or connections to help find the best way forward.

>85 Individuals representing ~65 organizations attending our RARE Entrepreneur Boot Camp since its 2017 inception

~195 Number of meetings our CEO and CMO have had since 2020 with families, patient groups and companies to share their expertise and provide drug development advice

For general inquiries about Ultragenyx, contact info@ultragenyx.com

For questions relating to Crysvita®, Mepsevii®, Dojolvi®, or Evkeeza®, contact Medical Information at medinfo@ultragenyx.com

For patient advocacy questions, contact patientadvocacy@ultragenyx.com

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