ultragenyx pharmaceutical

Transforming good science into great medicine for rare genetic diseases

Headquarters
Novato, CA

Founded
2010

Publicly-traded Company
Since 2014
NASDAQ: RARE

Employees
540+ on four continents
North America, South America, Europe, and Asia

Company Overview

- Biopharmaceutical company committed to developing novel medicines for patients living with rare and ultra-rare genetic diseases with no approved therapies.
- Led by individuals with decades of experience in all aspects of biopharmaceutical development and commercialization.
- Two approved therapies and three compounds in clinical development for rare and serious diseases using small molecules, biologics, mRNA, and gene therapy.

FDA-Approved and Marketed Medicines

Mepsevii™
(vestronidase alfa-vjbk)
injection, for intravenous use

CRYSVITA®
burosumab-twza
Injection for subcutaneous use

Pipeline

Diverse Clinical and Preclinical Pipeline

	Candidate	Description	Stage -2 to 0 / IND / Phase 1 / Phase 2 / Phase 3 / Filed	Est'd Patients in Dev. World
CLINICAL	Burosumab (KYOWA KIRIN)	*Anti-FGF23 monoclonal antibody*	XLH — *FDA Approved*	~48,000
			TIO	~2,000-4,000
	Mepsevii	*Enzyme replacement*	MPS 7 — *FDA Approved*	~200
	UX007	*Substrate replacement*	LC-FAOD	~8,000-14,000
			Glut1 DS	~12,000-28,000
	DTX301	*AAV8-OTC Gene Transfer*	OTC	~10,000
IND READY	DTX401	*AAV8-G6Pase Gene Transfer*	GSDIa	~6,000
	UX004	*rhPPCA ERT*	Galactosialidosis	~200
TRANSLATIONAL RESEARCH	DTX201	*AAV-FVIII Gene Transfer*	Hemophilia A	~144,000
	DTX701	*AAV-ATP7B Gene Transfer*	Wilson Disease	>50,000
	UX068	*Substrate replacement*	Creatine Transporter Deficiency	~10,000-50,000
	Arc-#1	*mRNA/ Lipid*		>20,000
	UX001P	*Substrate replacement*	GNE Myopathy	~2,000
	UX053	*mRNA/ Lipid*	GSDIII	>10,000
	Undisclosed	*Substrate replacement*	FSHD	>50,000
	DTX501	*AAV8-PAH Gene Transfer*	PKU	~50,000
	DTX701	*AAV8-ASS1 Gene Transfer*	Citrulinemia type I	~2,000

Small Molecule | Protein biologic | RNA | Gene Therapy

Please visit ultragenyx.com and ultrarareadvocacy.com for more information

"I've been fortunate to be present when the first patients were successfully treated with new medications for conditions that previously had no therapies. Those moments have stayed with me. Our goal at Ultragenyx is to create those moments for more patients."

– Dr. Emil Kakkis, CEO and President

Management

Emil D. Kakkis, M.D., Ph.D.
Chief Executive Officer and President

Shalini Sharp
Chief Financial Officer and Executive Vice President

Jayson Dallas, M.D.
Chief Commercial Officer and Executive Vice President

Camille Bedrosian, M.D.
Chief Medical Officer and Executive Vice President

Dennis Huang
Chief Technical Operations Officer and Executive Vice President

Thomas Kassberg
Chief Business Officer and Executive Vice President

Karah Parschauer
General Counsel and Executive Vice President

John Pinion
Chief Quality Operations Officer and Executive Vice President, Translational Sciences

Strategic Partnerships

Baylor Research Institute, Bayer, Kyowa Hakko Kirin, St. Louis University, REGENXBIO, St. Jude Children's Research Hospital, Takeda Pharmaceuticals, University of Pennsylvania

Commitment to Patients

- **Our purpose is to be heroes to our patients** by bringing them novel therapies for the treatment of rare and ultra-rare diseases. Everyone at Ultragenyx dedicates themselves to taking on challenges, targeting untreated diseases, and finding options for those who don't have any.
- **We collaborate closely with patient advocacy organizations** to raise awareness and help provide education and support to individuals and families affected by these disorders.
- **Ultragenyx contributes health-related and educational grants** to more than 55 local, state and national non-profits, patient organizations and university programs in 20 states.