

Company overview:

Orchard Therapeutics is a leading global fully integrated commercial stage biotech company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies.

Orchard is privately-held. In its latest, Series B funding round (Dec 2017), the company raised \$110M. Orchard was named a Fierce 15 Company by FierceBiotech in 2016.

Leadership team

- **Mark Rothera:** President & CEO
- **John Cerio:** SVP, Global Head of Human Resources
- **Stewart Craig:** Chief Manufacturing Officer
- **Anne Dupraz-Poiseau:** Chief Regulatory Officer
- **Bobby Gaspar:** Chief Scientific Officer
- **John Ilett:** General Counsel & Company Secretary
- **Nicolas Koebel:** Chief of Staff & Head of Program Management
- **Jason Meyenburg:** Chief Commercial Officer
- **Adrien Lemoine:** SVP Business Development & Alliance Management
- **Andrea Spezzi:** Chief Medical Officer
- **Frank Thomas:** Chief Financial Officer & Chief Business Officer

Mission

To transform the lives of **patients** with rare disorders through innovative gene therapies

Vision

To become a global, fully integrated biotech transforming the lives of patients with rare diseases through innovative gene therapies

Contact information

<http://www.orchard-tx.com>

Company offices:

- 2 Seaport Lane, Boston, MA 02210 (US); global commercial & administrative hub
- 108 Cannon Street, EC4N 6EU London (UK); R&D and EU hub
- 1118 Chess Drive, Foster City, CA 94494; manufacturing site
- 1360 O'Brien Drive, Suite A, Menlo Park, CA 94025 (US); manufacturing site

Orchard Therapeutics pipeline:

	In-vitro PoC	Animal PoC	IND/CT-enabling preclinical studies	Clinical PoC	Registrational trial	Commercialization
Primary immune deficiencies (PIDs) franchise						
Strimvelis	Adenosine deaminase severe combined immunodeficiency (ADA-SCID)					
OTL-101	Adenosine deaminase severe combined immunodeficiency (ADA-SCID)					
OTL-103	Wiskott-Aldrich syndrome (WAS)					
OTL-102	X-linked chronic granulomatous disease (X-CGD)					
Other PID	Undisclosed					
Research PID	Undisclosed					
Inherited metabolic diseases (IMDs) franchise						
OTL-200	Metachromatic leukodystrophy					
OTL-201	Mucopolysaccharidosis type IIIA					
OTL-202	Mucopolysaccharidosis type IIIB					
Other IMDs	Undisclosed					
Other						
OTL-300	Beta-thalassemia					

Orchard's portfolio of autologous *ex vivo* gene therapies has demonstrated sustained clinical benefit in over 132 patients across five disease areas.

These programs include Strimvelis, the first autologous *ex vivo* gene therapy approved by the EMA in 2016, 3 programs in advanced registrational studies in MLD (metachromatic leukodystrophy), WAS (Wiskott Aldrich syndrome) and ADA-SCID (adenosine deaminase severe combined immunodeficiency), 2 other clinical programs in X-CGD (X-linked chronic granulomatous disease) and beta-thalassemia, as well as an extensive preclinical pipeline.

Academic partnerships

Orchard's pipeline is the result of over two decades of academic research and partnerships with world-leading institutions in gene therapy, including:

- ◆ The University of California Los Angeles: **Prof. Donald Kohn**
- ◆ Boston Children's Hospital: **Prof. Alessandra Biffi; Prof. David Williams**
- ◆ University College London, Great Ormond Street Hospital: **Prof. Bobby Gaspar; Prof. Adrian Thrasher**
- ◆ The University of Manchester and Central Manchester University Hospitals: **Prof. Brian Bigger; Dr. Simon Jones; Prof. Rob Wynn**
- ◆ Telethon Institute of Gene Therapy and Ospedale San Raffaele: **Prof. Luigi Naldini; Prof. Alessandro Aiuti**

Board of Directors

- ◆ **Benjamin Auspitz**, Partner, F-Prime Capital (Chairman)
- ◆ **Marc Dunoyer**, AstraZeneca
- ◆ **Bobby Gaspar**, CSO Orchard Therapeutics
- ◆ **Alex Pasteur**, Partner, F-Prime Capital
- ◆ **Mark Rothera**, CEO Orchard Therapeutics
- ◆ **Charlie Rowland**, Board member Blueprint Medicines, Nabriva Therapeutics, Viking Therapeutics, Psioxus Therapeutics
- ◆ **Simone Song**, Partner ORI Capital

Investor base

Orchard Therapeutics has a global investor base, including:

- ◆ COWEN Healthcare Investments
- ◆ F-Prime Capital
- ◆ ORI Capital
- ◆ Temasek Holdings
- ◆ UCL Technology Funds