UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
WASHINGTON, D.C. 20549

FORM 8-K

CURRENT REPORT

Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934

Date of Report (Date of earliest event reported): **January 13, 2020**

ORCHARD THERAPEUTICS PLC
(Exact name of Registrant as Specified in Its Charter)

England and Wales	**001-38722**	**Not Applicable**
(State or Other Jurisdiction of Incorporation)	(Commission File Number)	(IRS Employer Identification No.)

108 Cannon Street
London EC4N 6EU
United Kingdom
(Address of Principal Executive Offices; Zip Code)

Registrant's Telephone Number, Including Area Code: **+44 (0) 203 808 8286**

Not Applicable
(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions:

☐ Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)

☐ Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)

☐ Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))

☐ Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Trading Symbol(s)	Name of each exchange on which registered
American Depositary Shares, each representing one ordinary share, nominal value £0.10 per share	ORTX	The Nasdaq Global Select Market

Indicate by check mark whether the registrant is an emerging growth company as defined in Rule 405 of the Securities Act of 1933 (§ 230.405 of this chapter) or Rule 12b-2 of the Securities Exchange Act of 1934 (§ 240.12b-2 of this chapter).

Emerging growth company ☐

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Item 2.02 Results of Operations and Financial Condition.

On January 13, 2020, Orchard Therapeutics plc (the "Company") issued a press release announcing a preliminary unaudited estimate of its cash and investments as of December 31, 2019 (the "Financial Information"). A copy of the press release is attached as Exhibit 99.1 to this current report on Form 8-K (the "Report"). The Financial Information is unaudited and does not present all information necessary for an understanding of the Company's financial condition as of December 31, 2019 and its results of operations for the three or twelve months ended December 31, 2019.

The Financial Information contained in Item 2.02 of this Report and Exhibit 99.1 attached hereto shall not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended (the "Exchange Act"), or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act of 1933, as amended (the "Securities Act"), or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 7.01 Regulation FD Disclosure.

The Company intends to participate in the 38th Annual J.P. Morgan Healthcare Conference during the week of January 13, 2020, including holding various investor and analyst meetings and presenting on January 14, 2020 at 11:30 a.m. Pacific Time. A copy of the Company's slide presentation is attached as Exhibit 99.2 to this Report. The Company undertakes no obligation to update, supplement or amend the materials attached hereto as Exhibit 99.2.

The information contained in Item 7.01 of this Report and Exhibit 99.2 attached hereto shall not be deemed "filed" for purposes of Section 18 of the Exchange Act, or otherwise subject to the liabilities of that section, nor shall it be deemed incorporated by reference in any filing under the Securities Act or the Exchange Act, except as expressly set forth by specific reference in such filing.

Item 8.01 Other Events.

On January 13, 2020, the Company issued a press release announcing its 2020 strategic priorities.

A copy of the press release is attached as Exhibit 99.1 to this Report and is incorporated herein by reference.

Item 9.01 Financial Statements and Exhibits.

(d) Exhibits.

Exhibit Number	Description
99.1	Press release dated January 13, 2020
99.2	Presentation of Orchard Therapeutics plc

SIGNATURES

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

ORCHARD THERAPEUTICS PLC

Date: January 13, 2020

By: /s/ Frank E. Thomas
Frank E. Thomas
Chief Financial Officer and Chief Operating Officer

Orchard Therapeutics Highlights 2020 Strategic Priorities

Commercial Preparations on Track for Potential 2020 EU Launch of OTL-200
for Metachromatic Leukodystrophy (MLD) with U.S. Regulatory Filing Expected Late 2020 / Early 2021

Initiation of Rolling U.S. Regulatory Filing for OTL-101 (ADA-SCID) Planned for 1H 2020;
U.S. and EU Regulatory Filings of OTL-103 (WAS) Expected in 2021

MPS-I and MPS-IIIA Proof-of-Concept Clinical Trials Ongoing with Additional Data Expected in 2020

New Research and Discovery Initiatives Expand Portfolio's Potential into Larger Neurodegenerative Diseases

$325M in Cash and Investments to Support Execution on Strategic Priorities in 2020 and Beyond

BOSTON and LONDON, January 13, 2020 (GLOBE NEWSWIRE) – Orchard Therapeutics (Nasdaq: ORTX), a global gene therapy leader, today outlined the company's strategic priorities and recent progress in conjunction with its attendance at the 38th Annual J.P. Morgan Healthcare Conference in San Francisco.

Mark Rothera, Orchard's president and chief executive officer, will present a business overview on Tuesday, January 14, 2020 at 11:30 a.m. PT that will be webcast live at ir.orchard-tx.com. He will summarize the company's strong fundamentals and differentiated approach to gene therapy utilizing gene-corrected hematopoietic stem cells (HSCs), with a special focus on the launch strategy for OTL-200 in metachromatic leukodystrophy (MLD) and newly announced research and discovery initiatives.

"2020 has the potential to be a watershed year for Orchard as we work to bring the benefits of our gene therapy approach and expertise to patients," Rothera said. "We are preparing diligently for the anticipated EU regulatory approval of OTL-200 for MLD while building the commercial team and executing our go-to-market strategy that will help ensure broad patient access. At the same time, we are readying our first regulatory filings in the U.S. and advancing multiple clinical-stage programs through important milestones, while also exploring the potential for gene-corrected HSCs in a broader range of severe disorders, including non-rare indications. With an unrelenting focus on execution, our organization is highly motivated to bring these investigational therapies to patients around the world."

2020 Corporate Priorities
Orchard has outlined the following three high-level corporate goals for 2020:

1. Obtain approval for and launch OTL-200 for the treatment of MLD in Europe and prepare for a biologics license application (BLA) filing in the U.S.;
2. Advance two registrational programs in primary immune deficiencies toward regulatory filings; and
3. Investigate the potential of our *ex vivo* HSC gene therapy platform approach in a broad set of neurodegenerative diseases and other new therapeutic areas, including ongoing proof-of-

concept clinical trials in mucopolysaccharidosis type I (MPS-I) and mucopolysaccharidosis type IIIA (MPS-IIIA).

Obtain Approval for and Launch OTL-200 for MLD in Europe
In preparation for a potential European approval in the second half of 2020, Orchard plans to establish the global infrastructure needed to support awareness and adoption of OTL-200. Orchard is putting in place a focused commercial team to serve as the backbone for the potential launch of OTL-200, as well as future product launches. A BLA filing for OTL-200 in the U.S. is planned for late 2020 or early 2021.

- Activities are underway to drive timely patient identification and access, including disease awareness, genetic testing and newborn screening pilots.
- The company continues to qualify treatment centers with specialized expertise in transplant and disease area knowledge across key geographies.
- The commercial supply chain for OTL-200 is being established, with the capacity and logistics to support an anticipated launch.
- The company has received stakeholder input on its value philosophy, as well as a set of guiding principles that will underpin Orchard's pricing and market access strategy.

Advance Two Registrational Programs Toward Upcoming Regulatory Filings
In support of its lead primary immune deficiency programs:

- The company plans to initiate a rolling BLA filing in the U.S. for OTL-101 in adenosine deaminase severe combined immunodeficiency (ADA-SCID) in the first half of 2020 with anticipated completion of the filing within 12 months.
- The company anticipates BLA and MAA regulatory filings for OTL-103 in Wiskott-Aldrich Syndrome (WAS) in the U.S. and EU in 2021.

Investigate the Potential of ex vivo HSC Gene Therapy in Additional Neurodegenerative Diseases and New Therapeutic Areas
Orchard has entered into an agreement with Dr. Alessandra Biffi, a leading expert in gene therapy, chair of the pediatric hematology, oncology and stem cell transplant division at Padua University and co-director of the gene therapy program at Dana Farber/Boston Children's Cancer and Blood Disorders Center, to support the expansion of its portfolio into additional areas of critical need for patients, including new programs for rare and non-rare neurodegenerative diseases. The company has also initiated in-house discovery programs to explore the application of *ex vivo* HSC gene therapy in other therapeutic areas.

Dr. Biffi commented, "Developing gene therapies for diseases with central nervous system involvement, such as neurometabolic and other neurodegenerative diseases, has been a challenge in the field. I am thrilled to partner with Orchard to continue development of this innovative gene therapy technology and, together, look forward to advancing potentially transformative treatments for these devastating diseases."

As part of the agreement, Dr. Biffi will serve as senior scientific advisor of neurometabolic diseases at Orchard. Dr. Biffi's previous role at the San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget) included the discovery and initiation of the OTL-200 program for MLD and the OTL-203 program for MPS-I.

In addition, Orchard plans to continue to advance the following clinical-stage neurometabolic programs currently underway in MPS-I and MPS-IIIA:

- The OTL-203 proof-of-concept clinical trial in MPS-I, which is being conducted at SR-Tiget, has reached its initial enrollment target of eight study participants. One-year follow-up results for the first eight patients, including the primary endpoints, are anticipated in 2021, with interim data planned for presentations at medical conferences during 2020.
- Enrollment has initiated in a proof-of-concept clinical trial for OTL-201 in MPS-IIIA, conducted by the Royal Manchester Children's Hospital. The trial is expected to enroll up to five patients in 2020 with interim data expected in 2020 and 2021.

Key 2019 Achievements
Orchard achieved each of its 2019 corporate milestones, with key achievements highlighted below.

- **MLD European MAA submission:** The Marketing Authorization Application (MAA) for OTL-200 for MLD was filed and accepted for review by the European Medicines Agency (EMA) in November 2019, ahead of previous guidance.
- **Cryopreserved gene therapy formulations:** Similar engraftment profiles have been observed between the cryopreserved and fresh formulations of OTL-200 for MLD and OTL-101 for ADA-SCID, which represents an important achievement toward the potential approvals of these investigational gene therapies and a key step toward global patient availability.
- **WAS registrational data set:** The registrational trial for OTL-103 for WAS met its key primary and secondary endpoints (n=8 at three years), including the elimination of severe bleeding episodes and a significant reduction in the frequency of severe infections.
- **MPS-I global license:** Orchard signed an exclusive license with Fondazione Telethon and Ospedale San Raffaele in Milan, Italy, for a clinical-stage HSC gene therapy program — OTL-203, a treatment for MPS-I that has shown promising early data in an ongoing proof-of-concept clinical trial.

Cash Guidance
The company ended 2019 with approximately $325 million of cash and investments. The company expects that its cash, cash equivalents and marketable securities as of December 31, 2019 will enable the company to fund its currently anticipated operating expenses and capital expenditure requirements into the second half of 2021, which includes the capital investment required to build-out a new manufacturing facility operated by Orchard in Fremont, CA.

Presentation at 38th Annual J.P. Morgan Healthcare Conference
Orchard will webcast its corporate presentation from the 38th Annual J.P. Morgan Healthcare Conference in San Francisco on Tuesday, January 14, 2020 at 11:30 a.m. PT. A live webcast of the presentation will be available under "News & Events" in the Investors & Media section of the company's website at orchard-tx.com. A replay of the webcast will be archived on the Orchard website following the presentation.

About Orchard

Orchard Therapeutics is a global gene therapy leader dedicated to transforming the lives of people affected by rare diseases through innovative, potentially curative gene therapies. Our *ex vivo* autologous gene therapy approach harnesses the power of genetically-modified blood stem cells and seeks to permanently correct the underlying cause of disease in a single administration. The company has one of the deepest gene therapy pipelines in the industry and is advancing seven clinical-stage programs across multiple therapeutic areas where the disease burden on children, families and caregivers is immense and current treatment options are limited or do not exist, including inherited neurometabolic disorders, primary immune deficiencies and blood disorders.

Orchard has its global headquarters in London and U.S. headquarters in Boston. For more information, please visit www.orchard-tx.com, and follow us on Twitter and LinkedIn.

Availability of Other Information About Orchard

Investors and others should note that Orchard communicates with its investors and the public using the company website (www.orchard-tx.com), the investor relations website (ir.orchard-tx.com), and on social media (twitter.com/orchard_tx and www.linkedin.com/company/10276396), including but not limited to investor presentations and investor fact sheets, U.S. Securities and Exchange Commission filings, press releases, public conference calls and webcasts. The information that Orchard posts on these channels and websites could be deemed to be material information. As a result, Orchard encourages investors, the media, and others interested in Orchard to review the information that is posted on these channels, including the investor relations website, on a regular basis. This list of channels may be updated from time to time on Orchard's investor relations website and may include additional social media channels. The contents of Orchard's website or these channels, or any other website that may be accessed from its website or these channels, shall not be deemed incorporated by reference in any filing under the Securities Act of 1933.

Forward-Looking Statements

This press release contains certain forward-looking statements about Orchard's strategy, future plans and prospects, which are made pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995. Such forward-looking statements may be identified by words such as "anticipates," "believes," "expects," "plans," "intends," "projects," and "future" or similar expressions that are intended to identify forward-looking statements. Forward-looking statements include express or implied statements relating to, among other things, the company's business strategy and goals, the therapeutic potential of Orchard's product candidates, including the product candidate or candidates referred to in this release, Orchard's expectations regarding the timing of regulatory submissions for approval of its product candidates, including the product candidate or candidates referred to in this release, the timing of interactions with regulators and regulatory submissions related to ongoing and new clinical trials for its product candidates, the timing of announcement of clinical data for its product candidates and the likelihood that such data will be positive and support further clinical development and regulatory approval of these product candidates, the likelihood of approval of such product candidates by the applicable regulatory authorities, and the company's financial condition and cash runway into the second half of 2021. These statements are neither promises nor guarantees and are subject to a variety of risks and uncertainties, many of which are beyond Orchard's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. In particular, the risks and uncertainties include, without limitation: the risk that any one or more of Orchard's product candidates, including the product candidate or candidates referred to in this release, will not be approved, successfully developed or commercialized, the risk of cessation or delay of any of Orchard's ongoing or planned clinical trials, the risk that prior results, such as signals of safety, activity or

durability of effect, observed from preclinical studies or clinical trials will not be replicated or will not continue in ongoing or future studies or trials involving Orchard's product candidates, the delay of any of Orchard's regulatory submissions, the failure to obtain marketing approval from the applicable regulatory authorities for any of Orchard's product candidates, the receipt of restricted marketing approvals, and the risk of delays in Orchard's ability to commercialize its product candidates, if approved. Given these uncertainties, the reader is advised not to place any undue reliance on such forward-looking statements.

Other risks and uncertainties faced by Orchard include those identified under the heading "Risk Factors" in Orchard's annual report on Form 20-F for the year ended December 31, 2018, as filed with the U.S. Securities and Exchange Commission (SEC) on March 22, 2019, as well as subsequent filings and reports filed with the SEC. The forward-looking statements contained in this press release reflect Orchard's views as of the date hereof, and Orchard does not assume and specifically disclaims any obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

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Exhibit 99.2

Orchard therapeutics

Dedicated to Transforming the Lives of Patients Through Innovative Gene Therapies

January 13 - 15, 2020

Forward Looking Statements

Certain information set forth in this presentation and in statements made orally during this presentation contains "forward-looking statements". Except for statements of historical fact, information contained herein constitutes forward-looking statements and includes, but is not limited to, the Company's expectations regarding: (I) the safety and efficacy of its product candidates; (II) the expected development of the Company's business and product candidates; (III) the timing of regulatory submissions for approval of its product candidates; (IV) the timing of interactions with regulators and regulatory submissions related to ongoing and new clinical trials for its product candidates; (V) the timing of announcement of clinical data for its product candidates and the likelihood that such data will be positive and support further clinical development and regulatory approval of these product candidates; (VI) the likelihood of approval of such product candidates by the applicable regulatory authorities; (VII) execution of the Company's vision and growth strategy, including with respect to global growth; and (VIII) projected financial performance and financial condition, including the sufficiency of the Company's cash and cash equivalents to fund operations in future periods and future liquidity, working capital and capital requirements. The words "may," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "potential," "continue," and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. Forward-looking statements are provided to allow investors the opportunity to understand management's beliefs and opinions in respect of the future so that they may use such beliefs and opinions as one factor in evaluating an investment.

These statements are neither promises nor guarantees of future performance. Such forward-looking statements necessarily involve known and unknown risks and uncertainties, which may cause actual performance and financial results in future periods to differ materially from any projections of future performance or result expressed or implied by such forward-looking statements. You are cautioned not to place undue reliance on forward-looking statements. These statements are subject to a variety of risks and uncertainties, many of which are beyond the Company's control, which could cause actual results to differ materially from those contemplated in these forward-looking statements. For additional disclosure regarding these and other risks faced by the Company, see the disclosure contained in the Company's public filings with the U.S. Securities and Exchange Commission (the "SEC"), including in the Company's annual report on Form 20-F filed with the SEC on March 22, 2019, as well as subsequent filings and reports filed with the SEC. These forward-looking statements speak only as of the date of this presentation. The Company undertakes no obligation to publicly update or revise any forward-looking statements, whether as a result of new information, future events or otherwise, except as may be required by law.

Orchard therapeutics

Global gene therapy company

Dedicated to transforming the lives of patients with rare diseases

Focused today on *ex-vivo* autologous HSC gene therapy

Orchard therapeutics

THE ORCHARD THERAPEUTICS STORY

Strong Momentum – Transformative Year Ahead

1 **Strong Fundamentals**

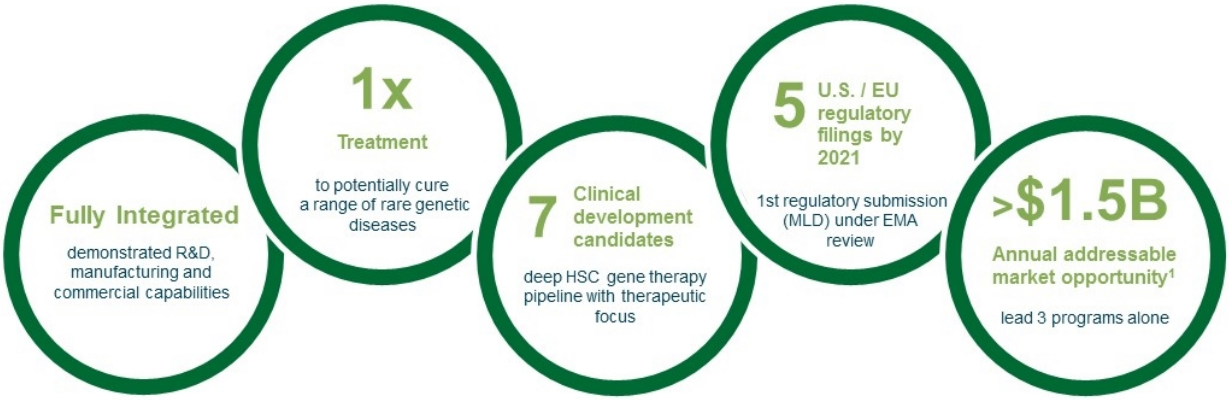
2 Powerful Platform Approach

3 Commercial Ready

4 Our Future

Orchard therapeutics

Compelling Fundamentals Driving Near and Long-term Growth



Highly leverageable **platform approach to gene therapy; potential to address numerous rare diseases**

Strong balance sheet (~$325M as of YE 2019) to fund the business into the second half of 2021

[1]Based on target label and pricing of recent gene therapy analogs, see slide 7-8 for additional detail

Orchard therapeutics

One of the Deepest Pipelines in Gene Therapy

Several additional research and preclinical programs under development

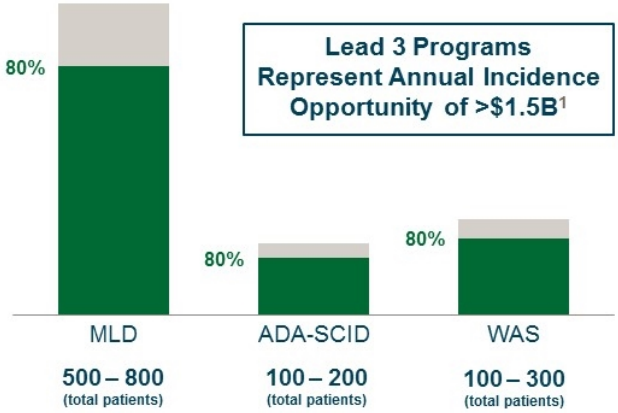
RPD Program with Rare Pediatric Disease Designation; eligible for a Priority Review Voucher I **BKT** Breakthrough Therapy Designation
PRIME Priority Medicine (PRIME) Designation I **RMAT** Regenerative Medicine Advanced Therapy

Orchard therapeutics

Building a Scalable Business in Rare Diseases

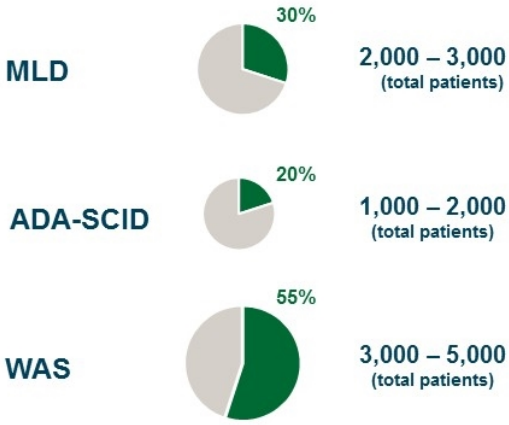
Ineligible
Eligible

Annual Incidence



Lead 3 Programs Represent Annual Incidence Opportunity of >$1.5B[1]

Prevalence



[1]Based on target label and pricing of recent gene therapy analogs

Incidence / prevalence figure estimates based on available literature, population data and in-house estimates, in countries where rare disease therapies are typically reimbursed. Higher incidence rates have been observed in certain populations with higher rates of consanguinity.

Epidemiology incidence references: MLD: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5027970/; SEG: https://ghr.nlm.nih.gov/condition/metachromatic-leukodystrophy
ADA-SCID; https://ghr.nlm.nih.gov/condition/adenosine-deaminase-deficiency#statistics SEG: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5911483/;
WAS: ASM. Annals of Saudi Medicine. Wiskott-Aldrich Syndrome; NIH Genetics Home Reference https://ghr.nlm.nih.gov/condition/wiskott-aldrich-syndrome.

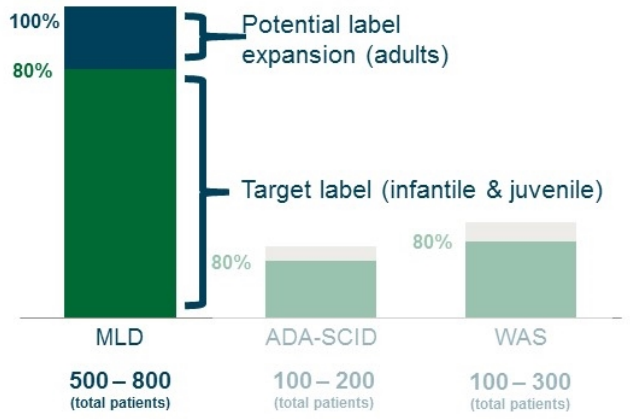
Orchard therapeutics

Initial Target Label Focusing on Pediatric MLD

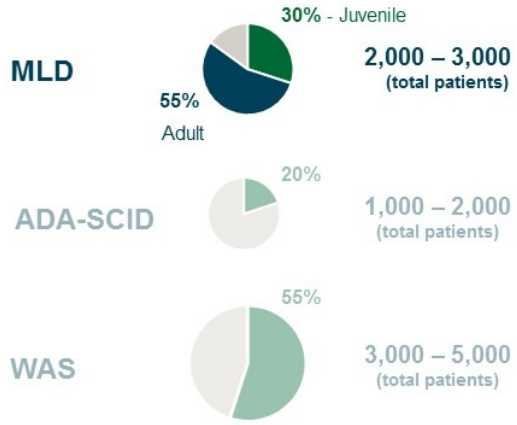
Potential label expansion opportunity in adult MLD

Ineligible
Eligible

Annual Incidence



Prevalence



[1]Based on target label and pricing of recent gene therapy analogs
Incidence / prevalence figure estimates based on available literature, population data and in-house estimates, in countries where rare disease therapies are typically reimbursed.
Higher incidence rates have been observed in certain populations with higher rates of consanguinity.
Epidemiology incidence references: MLD: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5027970/; SEG: https://ghr.nlm.nih.gov/condition/metachromatic-leukodystrophy
ADA-SCID; https://ghr.nlm.nih.gov/condition/adenosine-deaminase-deficiency#statistics SEG: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC5911483/;
WAS: ASM. Annals of Saudi Medicine. Wiskott-Aldrich Syndrome; NIH Genetics Home Reference https://ghr.nlm.nih.gov/condition/wiskott-aldrich-syndrome.

Orchard therapeutics

THE ORCHARD THERAPEUTICS STORY

Strong Momentum – Transformative Year Ahead

1 Strong Fundamentals

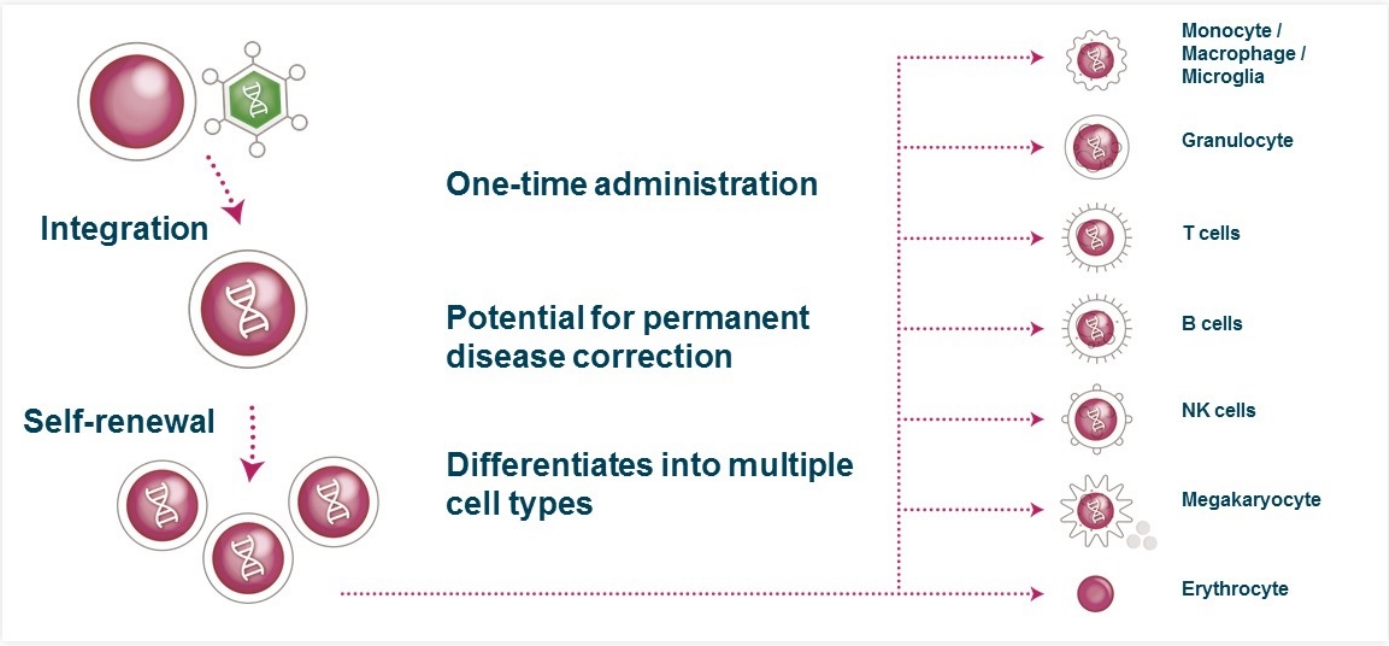
2 **Powerful Platform Approach**

3 Commercial Ready

4 Our Future

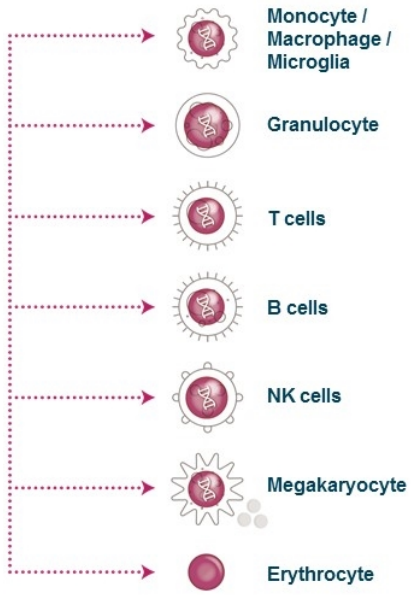
Orchard therapeutics

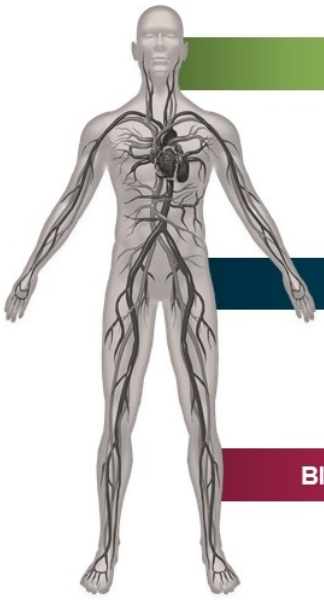
HSC Gene Therapy Offers a Highly Differentiated Approach



Orchard therapeutics

Potential to Correct Multiple Cell Types Addresses Many Rare Diseases





Central Nervous System

- MLD
- MPS-I
- MPS-IIIA
- MPS-IIIB

Immune System

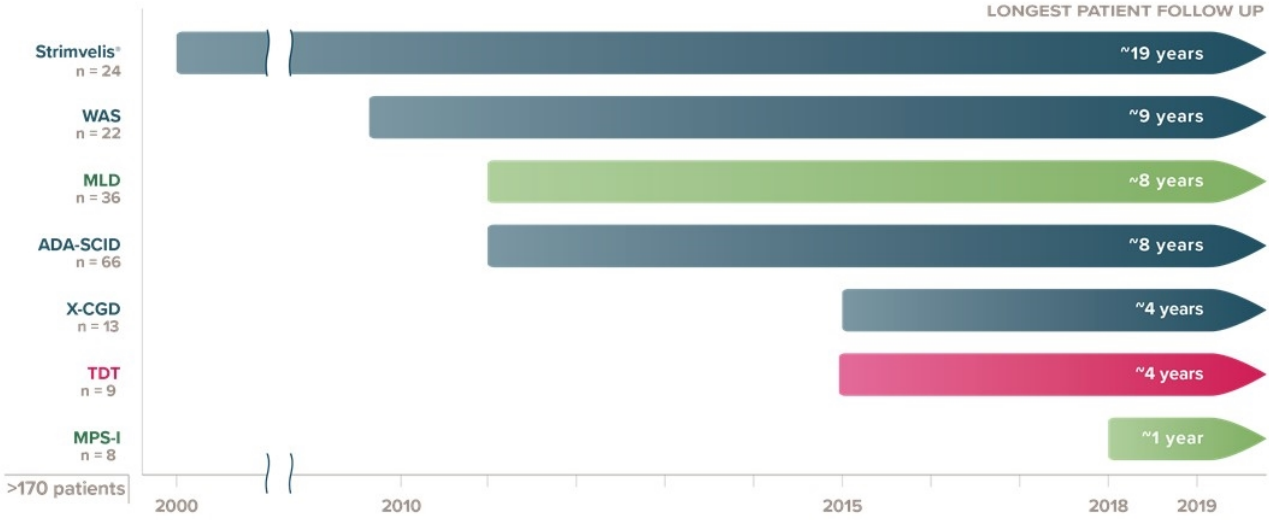
- ADA-SCID
- WAS
- X-CGD

Blood Cell and Platelet Lineage

- WAS
- TDT

Orchard therapeutics

Durability of Response Out to 18+ Years with > 170 Patients Treated



Patients treated in the development phase, including in clinical trials and under pre-approval access (defined as any form of pre-approval treatment outside of a company-sponsored clinical trial, including, but not limited to, compassionate use, early access, hospital exemption or special license).
Patient with longest Strimvelis® follow-up enrolled in registry study, with data available up to 19 years.
Data based on in-house data as of December 2019.

Data include all patients treated with CD34+ hematopoietic stem cells transduced ex vivo with vector of interest.

Orchard therapeutics

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Orchard therapeutics

Metachromatic Leukodystrophy is a Devastating, Rapidly Progressive Disease

Disease Snapshot

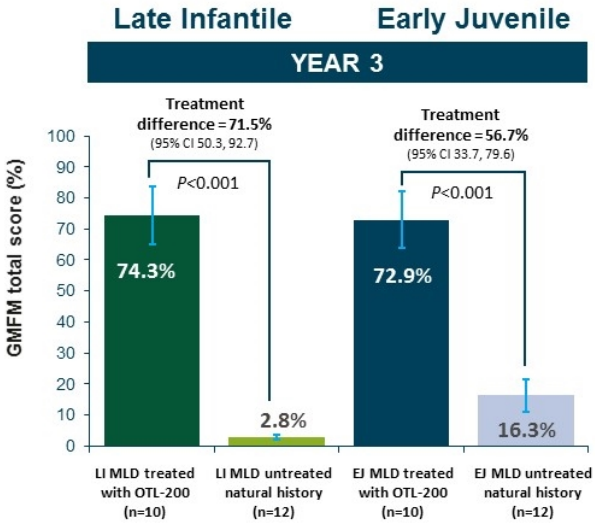
Age 5, pre-diagnosis

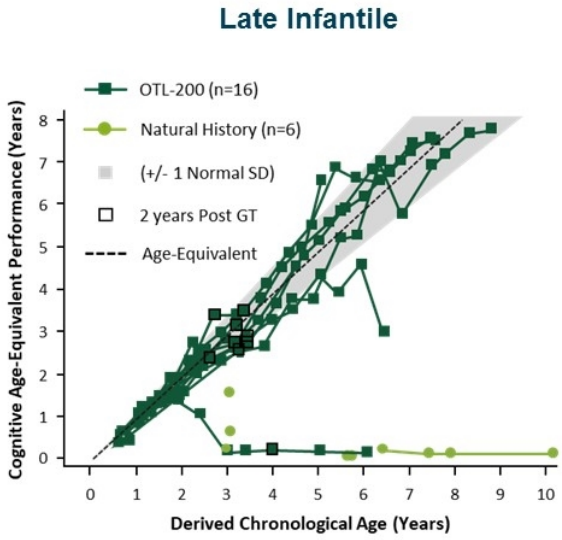
Age 9, advanced disease

- Fatal genetic CNS disorder
- Relentless loss of physical and cognitive function
- Presents on a spectrum with different ages of onset

Orchard therapeutics

Significantly Superior Motor and Cognitive Function Demonstrated vs. Natural History





Both LI and EJ patients achieved a statistically significant difference on the co-primary endpoint of improvement of >10% of the total GMFM score in treated subjects when compared to the Natural History cohort at Year 2, and these were maintained through Year 3. Note: vertical error bars are SE of the adjusted mean; P-values are from a two-sided 5% hypothesis test with null hypothesis of ≤ 10% difference ;CI, confidence interval; EJ, early juvenile; GMFM, gross motor function measurement; LI, late infantile; MLD, metachromatic leukodystrophy.

Cognitive Age-Equivalent at each visit has been derived as follows: For WPPSI and WISC: (DQp x Chronological Age)/100. For Bayley III: Cognitive Raw Scores have been compared to the tabulated values in the Bayley III manual to calculate Cognitive Age-Equivalent. For Bayley II: Cognitive Age-Equivalent is based on Mental Development Age as reported on the CRF. The Psychological Corporation. 2006.Bayley N. Bayley scales of infant and Toddler Development. Third Edition. San Antonio.

Integrated analysis presented September 4 2019 at SSIEM annual meeting

Orchard therapeutics

Implementing Commercial Strategy to Launch OTL-200 Globally

1 Patient ID and Diagnostics	• Disease awareness and diagnostic testing • Extensive newborn screening pilot testing efforts in EU and U.S.
2 Geographic Footprint	• Teams in place in key EU markets and U.S. • Expansion to Middle East, Turkey, LatAm and Asia over time • Qualifying leading centers with transplant and disease area experience
3 Global Supply Network	• Manufacturing hub to ship cryopreserved product globally • Inventory, capacity and logistics of supply for launch
4 Market Access	• Multi-stakeholder engagement • Gene therapy value determination • Option for flexible payment models

Orchard therapeutics

Multi-Pronged Approach to Identifying MLD Patients

Disease and Diagnostic Education

- Identify first symptoms and drive early suspicion
- KOL engagement and education of general pediatricians
- Peer to peer education

Diagnostic Initiatives

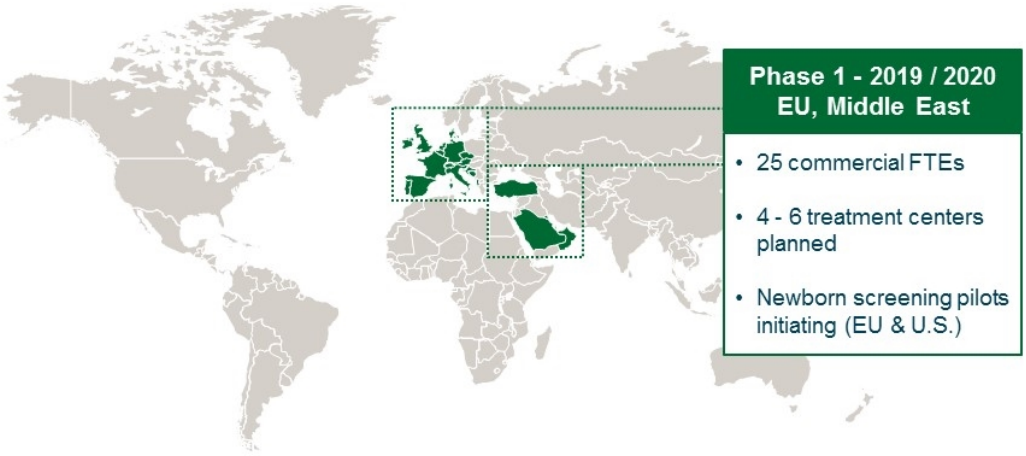
- Sponsor diagnostic tests in academic centers and targeted centers of care
- Goal to test within 14 days of early suspicion

Newborn Screening

- Assay developed
- EU and U.S. pilots planned to initiate in 2020
- Advocacy efforts at state / country level

Orchard therapeutics

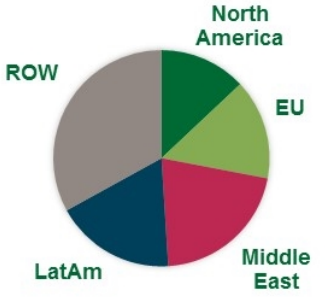
Establish Commercial and Operational Model: Building Teams and Infrastructure to Serve Patients Worldwide



Phase 1 - 2019 / 2020
EU, Middle East

- 25 commercial FTEs
- 4 - 6 treatment centers planned
- Newborn screening pilots initiating (EU & U.S.)

GEOGRAPHIC MLD PATIENT DISTRIBUTION (ESTIMATED)
2,000 – 3,000 PATIENTS



Penetration and reimbursement varies by market

Middle East includes GCC countries and Israel

Orchard therapeutics

Establish Commercial and Operational Model: Building Teams and Infrastructure to Serve Patients Worldwide

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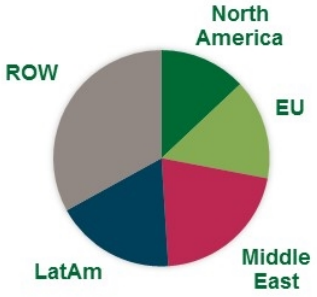

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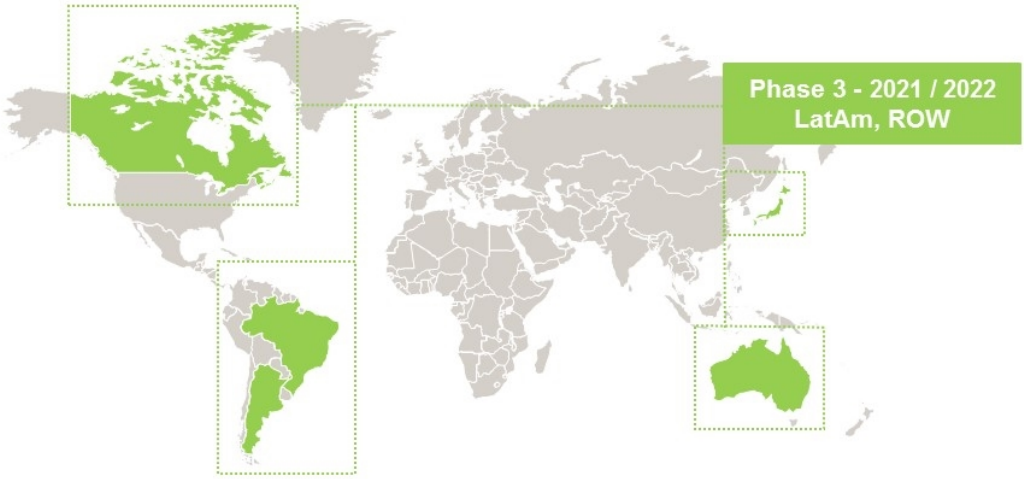

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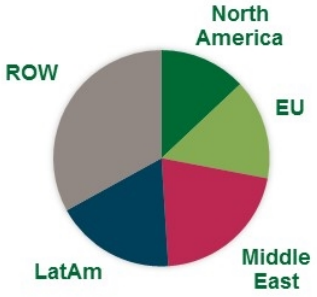

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Orchard therapeutics

Manufacturing in Place to Support Commercialization

NEAR TERM

CMO Infrastructure Established for Initial MLD Launch

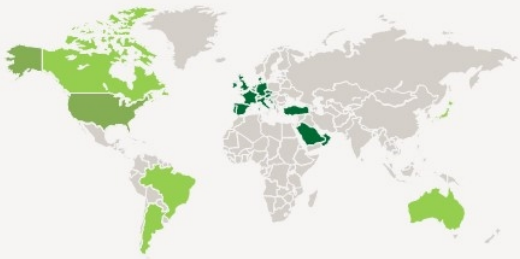
- MolMed producing drug product and vector
- Sufficient inventory being produced to meet expected launch demand
- Leveraging commercial process experience from Strimvelis

MOLMED

MID - LONG TERM

Invest in Own Manufacturing Facilities

- Continue partnerships with CMOs
- Orchard facility in Fremont, CA
  - Design complete
  - Construction planned to initiate in 2020
  - Goal to be operational by 2021



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Drive Global Access: Our Pricing Commitments

Shared value	Ensure patients, society and industry share in human and economic benefits of innovation – now and in the future
Shared risk	Stand behind therapeutic outcomes and be willing to tie payment to outcomes
Informed pricing	Use demonstrable, objective measures of value and the best available evidence to inform price
System-wide evolution	Support healthcare system to adapt to emergence of one-time, potential cures

Orchard therapeutics

THE ORCHARD THERAPEUTICS STORY

Strong Momentum – Transformative Year Ahead

1 Strong Fundamentals

2 Powerful Platform Approach

3 Commercial Ready

4 **Our Future**

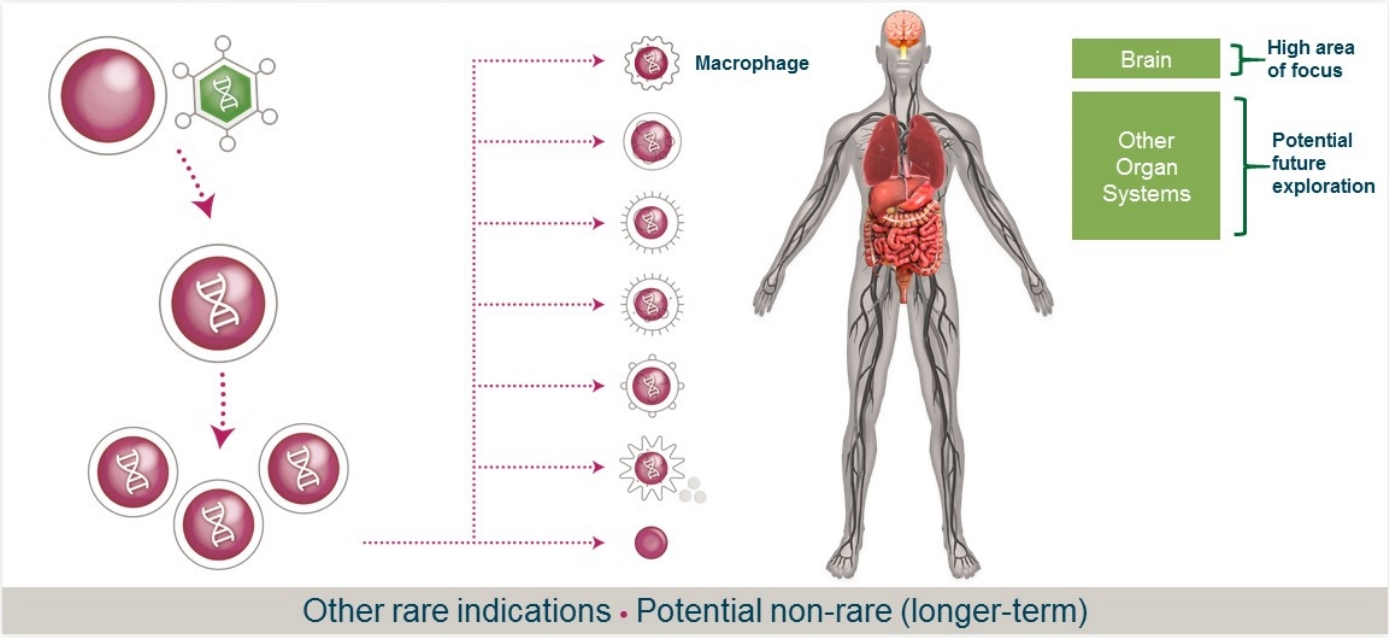
Orchard therapeutics

Investment to Scale and Innovate Across Our Business

Technology innovations		In-house capabilities
PRODUCT	PROCESS	
Transduction enhancers	Stable cell line	Manufacturing facility (expected operational in 2021)
Non-toxic conditioning	Closed automated systems	Research and discovery for new indications in 2020
HSC subsets		

Orchard therapeutics

Gene-corrected HSCs and Macrophage Progeny Can Address Brain and Other Organ System Disorders



Other rare indications • Potential non-rare (longer-term)

Literature references: Alessia Capotondo, Rita Milazzo, Letterio Salvatore Politi, Angelo Quattrini, Alessio Palini, Tiziana Plati, Stefania Merella, Alessandro Nonis, Clelia di Serio, Eugenio Montini, Luigi Naldini, and Alessandra Biffi, PNAS September 11, 2012 109 (37) 15018-15023; https://doi.org/10.1073/pnas.1205858109; Tissue macrophages: heterogeneity and functions, Siamon Gordon and Annette Plüddemann, BMC Biology 2017 15:53, 29 June 2017

Orchard therapeutics

Building on our Strength in Neurometabolic Disorders

Orchard therapeutics

Corporate Priorities & Expected Key Milestones

MLD	Obtain approval and launch OTL-200 for MLD in Europe in 2H 2020 Submit a BLA filing in late 2020 or early 2021
ADA-SCID	Initiate a rolling BLA in the U.S. for OTL-101 in 1H 2020 with completion within 12 months
WAS	Submit BLA and MAA filings for OTL-103 in 2021
MPS-I	Release interim data from POC study in 2020
MPS-IIIA	Enroll 5 patients in POC study, release interim data in 2020
Manufacturing	Initiate construction of in-house manufacturing facility in 2020

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