Rare Diseases Summit

2017 Final Report

In partnership with

ultragenyx pharmaceutical AUDENTES Fulcrum Therapeutics Springboard® ENTERPRISES

“The overall takeaway for me was extremely valuable and will undoubtedly help shape Wylder Nation's direction moving forward…I was able to connect with many people that I feel genuinely can and are willing to help me navigate this complicated space that is still very new to me.”

- Steven Laffoon, Wylder Therapeutics

“The Rare Diseases Summit was a tremendous experience. In small group sizes I was able to ask questions that were very specific to our field. The mentors at the Summit were amazing and offered their valuable input and advice without hesitation. It was a great experience.”

- Genine Winslow, Chameleon Biosciences

OVERVIEW

Emil Kakkis, Founder & CEO of Ultragenyx

Steven Laffoon (Wylder Therapeutics)

The 2017 **Rare Diseases Summit** was hosted by Ultragenyx Pharmaceutical and Springboard Enterprises along with partners Audentes Therapeutics and Fulcrum Therapeutics on November 15-17. Participants included entrepreneurs, experts in the field and parent advocates promoting and developing solutions for specific rare and ultra-rare diseases. This Summit was the first of what is planned to become an annual resource for entrepreneurs developing in the rare disease space.

The goal of the program, conceived by Ultragenyx's **Yael Weiss**, was to provide those seeking to commercialize rare disease solutions with a platform to connect with experts and peers. Her vision and drive brought together the foremost experts and entrepreneurs in the space to lead sessions on regulatory strategy, modality, development, legal structure, intellectual property, partnering and capital.

Matthew Patterson of Audentes, **Walter Kowtoniuk** of Fulcrum Therapeutics and **Emil Kakkis** of Ultragenyx joined a number of Springboard alumnae in sharing their experiences building rare disease and life science companies, offering participants invaluable insights on what they could expect on the road ahead.

Audrey Davidow (Pitt Hopkins Research Foundation)

Yael Weiss (Ultragenyx)

Tauhid Ali (Takeda)

PARTNERS AND SPONSORS

The 2017 Rare Diseases Summit was presented in partnership with **Ultragenyx Pharmaceutical**, **Springboard Enterprises**, **Audentes Therapeutics**, **Fulcrum Therapeutics** and with the support of Summit sponsors **BridgeBio**, **Cydan**, **Fenwick & West**, **Morgan Lewis**, **Precision for Medicine**, and **Takeda Pharmaceutical Company**.

Rare Diseases Summit Partners

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AGENDA

Day 1
The Summit opened with an orientation followed by interactive sessions during which the participants introduced themselves, their disease targets, goals and aspirations, the current status of their solutions and their challenges.

Sessions included:

- *Founding Stories & Company Introductions*
- *Building & Expanding the Core Team*
- *Corporate External Innovation & Accelerators*

Day 2
Breakout sessions for Gene Therapy and Small Molecules/Biologics solutions included interactive sessions on their milestones and development strategy and panel discussions addressing legal issues for early and growth stage companies.

Sessions included:

- *Building a Gene Therapy Company*
- *Modality*
- *Value Creating Milestones*
- *Development Strategy (IND Enabling Pre-Clinical Package, Choosing Endpoints, Manufacturing, Regulatory Strategy in Major Markets)*
- *Legal (Corporate Legal and Licensing, Intellectual Property)*

Day 3
A special presentation by Ultragenyx founder and CEO **Emil Kakkis** on the founding of Ultragenyx was also a celebration of the company's **first FDA approval** for MPS VII (Sly Syndrome). Panel discussions on partnering and sources of capital were featured.

Sessions included:

- *Strategic Partnering*
- *Sources of Capital & Investor Presentations*
- *Starting a Company: What does it take?*

Left to Right: Yael Weiss, Tom Kassberg (Ultragenyx), Walt Kowtoniuk (Fulcrum Therapeutics), and Neil Kumar (BridgeBio).

RARE DISEASES SUMMIT SPEAKERS

Tauhid Ali
Vice President, Tak-Celerator™
Takeda

Pamela Contag (Springboard Alumna)
CEO, BioEclipse Therapeutics

Mary Del Brady (Springboard Alumna)
Life Science Executive

Scott Galasinski
Senior Director, Translational Research, Ultragenyx

Graeme Fielder
Associate Director, Corporate Development, Audentes

Jennifer Friel Goldstein
Managing Director, Life Sciences & Healthcare, Silicon Valley Bank

Barbara Handelin (Springboard Alumna)
Founder, NovoKen

Mark Hayman
Partner, Morgan, Lewis & Bockius

Joshua Henderson
Vice President
Springboard Enterprises

Natalie Holles
COO, Audentes

Dennis Huang
Chief Technical Operations Officer, EVP, Ultragenyx

Emil Kakkis
Founder, President & CEO, Ultragenyx

Tom Kassberg
Chief Business Officer, Ultragenyx

Dione Kobayashi
Vice President, Pre-Clinical Translation, Cydan

Walter Kowtoniuk
Director, Strategy & Operations
Fulcrum

Neil Kumar
CEO & Founder, BridgeBio Pharma

Cori Leonard
SVP, Head of Regulatory Affairs
Ultragenyx

Christina MacDougall
Associate, Morgan, Lewis & Bockius

Amy Millman
President, Springboard Enterprises

Lucia Mokres
Clinical Development & Regulatory Strategy Executive

Mike Murtagh
Senior Director, Regulatory Affairs
Audentes

Bee Nguyen
Human Resources, Ultragenyx

Karah Parschauer
VP, General Counsel, Ultragenyx

Matthew Patterson
President & CEO, Audentes

Sue Preston (Springboard Alumna)
Consultant, x'Elas Biodevelopment

Stefano Quintini
Partner, Technology Transactions
Fenwick & West LLP

Mike Rossiter
Partner, Fenwick & West LLP

Shalini Sharp
Chief Financial Officer & EVP
Ultragenyx

Ali Skrinar
Executive Director, Clinical Outcomes Research and Evaluation, Ultragenyx

Sam Wadsworth
CSO, Dimension Therapeutics

Yael Weiss
VP Business Development, Ultragenyx

Lena Wu
CEO & President,
Co-Founder, Intabio

OVERVIEW OF ENTREPRENEURS

The participating entrepreneurs included parents, patient advocates, and researchers deeply invested in developing solutions for rare diseases. **Fourteen** entrepreneurs representing **twelve** companies and organizations joined us at the Ultragenyx headquarters in Novato, CA.

Disease Areas of Focus:

Acid Sphingomyelinase Deficiency (ASMD)
Adult Polyglucosan Body Disease
Batten Disease (CLN1)
Congenital monogenic diseases affecting metabolism in liver cells
Duchenne Muscular Dystrophy
Epidermolusis bullosa (EB)
Familiar Cardiomyopathy
Hemophilia A and B
Hereditary neuropathy with liability to pressure palsies (HNPP)
Huntington's disease
Limb Girdle Muscular Dystrophy 2D (Alpha-Sarcoglycanopathy)
Niemann-Pick Type A/B
Parry-Romberg Syndrome
Pitt Hopkins Syndrome
Progressive osseous heteroplasia (POH)
Sanfilippo Syndrome

Sean Ekins (Collaboration Pharmaceuticals) and Jill Wood (Phoenix Nest Biotech)

Tetsuro Wakatsuki (InvivoSciences)

THE ENTREPRENEURS

APBDRF
Jeff Levenson, Advisor

The Foundation works towards: Improving the diagnosis and identifying treatment(s) and cure(s) for APBD; Supporting individuals and families affected by the disease; Increasing awareness of the disease among health professionals and the public. Compounds that treat or cure the disorder are on the near horizon. Guaiacol is one such promising compound. A clinical trial is being proposed in Israel, and we need to move the US regulatory process forward so as to potentially be able to open a satellite arm or a parallel study for patients in the US. Because of the debilitating nature of the disorder many will find an overseas trip too arduous to participate.

Chameleon Biosciences
Genine Winslow, Founder, President, CEO, and Laura Corral

Chameleon Bio is a pre-clinical stage gene therapy company, developing a proprietary first in class vector platform technology for repeated dosing of systemic gene therapy. Chameleon's novel vectors deliver therapeutic proteins to specific tissues while reducing antigen specific immune responses to both the vector and the therapeutic protein. Chameleon is transforming gene therapy with repeat dosing for treatment of devastating diseases.

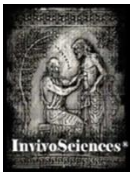
Collaborations Pharmaceuticals Inc.
Sean Ekins, CEO

Collaborations Pharmaceuticals Inc. (CPI) works with rare disease foundations and academic collaborators to develop treatments for rare or neglected infectious diseases. CPI actively seeks out scientists and projects which could benefit from STTR, SBIR funding and then in-license treatments that meet CPI success criteria. CPI operates as a quasi-virtual R&D start-up with a small lab and office on the NC State University Centennial Campus, while leveraging our collaborator's research infrastructure.

THE ENTREPRENEURS

Genogen Inc.
Nancy Mize, CEO, and Megan Montgomery

Genogen is a San Francisco Bay Area company developing regenerative medicine products. Our first product regenerates and repairs skin by turning on the body's own skin cells replacing the natural fat layer and elastin under the skin to repair underdeveloped areas of the face for the rare disease, Parry-Romberg Syndrome. We are currently conducting a human clinical study for proof of principle with 8 HIV+ subjects at the California Pacific Medical Center in San Francisco.



InvivoSciences
Tetsuro Wakatsuki, CSO

InvivoSciences, Inc. (IVS) is a drug discovery company with pre-clinical programs in heritable cardiomyopathies and cardio-oncology. IVS has been offering products and services to pharmaceutical, biotech companies, and research laboratories for their target discovery & validation and compound screening using 3D micro tissue-based phenotypic assay system. IVS also provides cardiac safety screening and *in silico* analysis following the new standards.

Myonexus Therapeutics
Bryan Barber, President

Myonexus Therapeutics is a clinical stage gene therapy company developing first ever treatments for Limb-girdle muscular dystrophy (LGMD) types 2D, 2B, 2E, 2L, and 2C based on research at Nationwide Children's Hospital, a leader in muscular dystrophy gene therapy discovery and translational research.

THE ENTREPRENEURS

NostoPharma
Jelena Jeremic, CSO

Nostopharma is a small, women-owned, Maryland-based pharma start-up developing formulation for application in rare diseases and post-surgical applications. Our first-to-launch indication is Progressive osseous heteroplasia (POH). In this rare pediatric condition, the lack of pharmacological treatment option is a big unmet medical need and we anticipate increased grant support and decreased development timeline. POH is an extremely rare disorder characterized by abnormal development of bone in areas of the body where bone is not normally present (heterotopic ossification).

Origami Therapeutics, Inc.
Beth Hoffman, President & CEO

Origami Therapeutics is a discovery stage start-up with a focus on neurological diseases. Using its core research platform, Origami has developed a proprietary screening platform to identify small molecules that "fix broken proteins" causally linked to disease. By targeting the underlying cause of disease and restoring normal function to mutated proteins, Origami plans to generate a pipeline of small molecule therapeutics that delay the onset and progression of neurodegenerative diseases.

Phoenix Nest Biotech
Jill Wood, CFO

Phoenix Nest, Inc. works with collaborators to develop treatments for Sanfilippo Syndrome. We have multiple potential products in our pipeline for MPS IIIB, IIIC and IIID.

THE ENTREPRENEURS

Pitt Hopkins Research Foundation
Audrey Davidow, President

The mission of the Pitt Hopkins Research Foundation (PHRF) is to support research dedicated to finding a treatment and an eventual cure of Pitt Hopkins syndrome and other similar disorders. Scientists at University of Alabama and University of North Carolina have created Pitt Hopkins Mouse models and neuronal stems cells and are testing them to see what drugs may help ameliorate symptoms. This year, scientists discovered not one, but two therapeutics that have reversed the symptoms of Pitt Hopkins and hope to bring these drugs to trial by the end of this year.

Propagenix Inc.
Sherry Challberg, COO & Co-Founder

Propagenix is a preclinical-stage platform technology company that is applying its unique and proprietary technology to both internal and partnered cell therapy programs. Our proprietary technology platform enables the generation in vitro of unprecedented quantities of certain essential cell types that can be used to replace damaged or defective cells in patients. Unlike cell therapies based upon the use of pluripotent ESCs or iPSCs, Propagenix's approach is simple, safe and cost-effective.

Wylder Therapeutics
Steven Laffoon, President & Founder

Wylder Therapeutics is a fully owned subsidiary of Wylder Nation Foundation, a 501(c)(3) non-profit organization formed to improve the lives of children diagnosed with a Lysosomal Storage Disease (LSD) by accelerating the discovery and development of treatment options. The company was formed to open up the opportunity for additional creative funding avenues outside of traditional non-profit fund raising to help advance our most promising research programs for Niemann-Pick Type A and similar LSDs.

SUMMIT PARTNERS

Ultragenyx Pharmaceutical is clinical-stage biopharmaceutical company committed to bringing to market novel products for the treatment of rare and ultra-rare diseases, with a focus on serious, debilitating genetic diseases. Founded in 2010, the company has rapidly built a diverse portfolio of product candidates with the potential to address disease for which the unmet medical need is high, the biology for treatment is clear, and for which there are no approved therapies.

Audentes Therapeutics is a biotechnology company committed to the development and commercialization of innovative new medicines for people with serious, rare muscle diseases. We are a passionate, energetic team driven by the goals of improving the lives of patients and building a successful business. We take pride in strong, global relationships with the patient, research, and medical communities.

Fulcrum Therapeutics is a biotechnology company developing precisions medicines to deliver a new future to patients and their families. Fulcrum's therapies are based on modulating gene regulation via control of genetic on and off switches of disease genes. Fulcrum, headquartered in Cambridge, MA, was launched by Third Rock Ventures in 2016 and named a "Fierce 15" company later that year.

Through its programs and global network, venture catalyst **Springboard Enterprises** partners with women-led technology, life sciences, and healthcare companies seeking funding or strategic partners and empowers the women leaders of those companies with access to a suite of relevant experts, services, and resources. Since 2000, it has created the leading global network of influencers, investors and innovators dedicated to helping women build scalable, sustainable businesses.

SUMMIT SPONSORS

BridgeBio is a clinical-stage biotechnology company developing novel, genetically targeted therapies to improve the lives of patients. The BridgeBio approach combines a traditional focus on drug development with a unique corporate model, allowing rapid translation of early stage science into medicines that treat rare diseases at their source. Founded in 2015 by a team of industry veterans who previously brought more than a dozen products to market, BridgeBio has built a portfolio of ten transformative drug programs that address rare diseases across oncology, cardiology, dermatology and endocrinology. The drugs are in various phases of development, from discovery to late clinical stage.

Cydan is the first orphan drug accelerator dedicated to advancing therapies that improve the lives of patients with rare genetic diseases. We are doing this because there are more than 7,000 rare diseases, with approximately 400 approved treatments. That's not good enough. We conceived of Cydan in 2012 and launched the company in 2013, assembling a team of drug hunters with extensive drug discovery, clinical development and business development experience. In early 2015, we launched our first new company – Vtesse – which is developing drugs for Niemann-Pick Disease Type C (NPC) and other rare, severe diseases with great unmet need. We are financed by leading life sciences investors, New Enterprise Associates, Pfizer Venture Investments, Lundbeckfond Ventures, Bay City Capital, Alexandria Venture Investments and Longitude Capital. The Cydan accelerator is based in Technology Square in Cambridge, MA.

The Fenwick & West Life Sciences Group is a multi-disciplinary team comprised of more than 60 attorneys who have grown up with the industry focused on advising many of the country's leading private and public life sciences companies. Our attorneys and patent agents bring to bear fully-integrated teams that provide critical legal support on licensing and partnering transactions, financings, corporate governance, securities offerings, buy and sellside M&A transactions, intellectual property strategy and complex litigation matters. Clients rely on the industry experience, judgment and broad knowledge base of our life sciences team to maximize their enterprise value and successfully navigate the legal challenges faced in today's highly competitive environment. For more information, please visit www.fenwick.com.

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Takeda

Takeda Pharmaceutical Company Limited is a global, research and development-driven pharmaceutical company committed to bringing better health and a brighter future to patients by translating science into life-changing medicines. Takeda focuses its R&D efforts on oncology, gastroenterology and central nervous system therapeutic areas plus vaccines. Takeda conducts R&D both internally and with partners to stay at the leading edge of innovation. New innovative products, especially in oncology and gastroenterology, as well as our presence in Emerging Markets, fuel the growth of Takeda. More than 30,000 Takeda employees are committed to improving quality of life for patients, working with our partners in health care in more than 70 countries.

For more information, visit www.takeda.com/news.

“I feel lucky to be part of such an inspirational sharing experience with Ultragenyx at the very beginning of my entrepreneurial journey. I learned a lot and will continue to pursue my dream of bringing cure for kids who currently have no hope. It was very inspirational!”

- Jelena Jeremic, NostoPharma

“I came away with a clear sense there was a lot of work to do to bring any of the projects to the clinic… An A-Z of everything you need to know to build a rare disease company.”

- Sean Ekins, Collaborations Pharmaceuticals Inc.

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