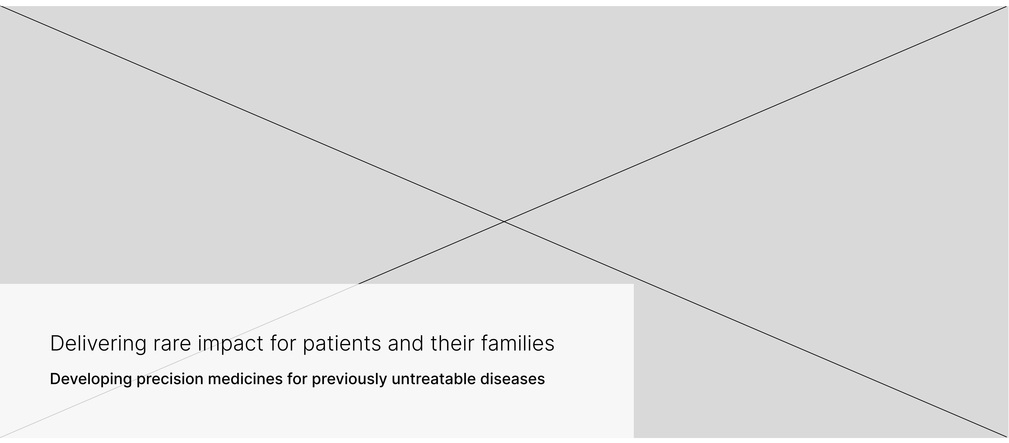

Delivering rare impact for patients and their families

Developing precision medicines for previously untreatable diseases

Rhythm Pharmaceuticals is a global commercial-stage biopharmaceutical company committed to developing transformational precision medicines for patients and their families living with rare neuroendocrine diseases.

We are defined by our ability to deliver rare impact. We develop precision medicines for previously untreatable or undertreated diseases and provide meaningful support for healthcare providers, patients, their families, and their communities.

Who We Are

We are a community-focused company that provides meaningful support for healthcare providers, patients, and their families.

Meet us →

Advancing Science

Rhythm is focused on delivering effective and meaningful solutions for rare neuroendocrine diseases.

See the science →

Our Pipeline

Innovative therapies for rare diseases

Learn more →

Our Passion for Helping Patients

We do much more than develop leading-edge therapies for rare diseases.

Here you can find **meaningful support for patients.**

See the support →

Want to work with us?

We are a fast-growing company that offers a collaborative environment where employees feel like their efforts and contributions truly matter.

See current opportunities →

Our news

More news →

2-8-2024

Rhythm Pharmaceuticals Announces New Employment Inducement Grants

Read more →

2-7-2024

Rhythm Pharmaceuticals Announces Positive Reimbursement Decision in Italy for IMCIVREE® (setmelanotide)...

Read more →

1-31-2024

Rhythm Pharmaceuticals to Report Fourth Quarter and Full Year 2023 Financial Results on Thursday, ...

Read more →

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222 Berkeley Street, 12th Floor
Boston, MA 02116

Using science to transform the lives of patients living with rare neuroendocrine diseases

At Rhythm, we are dedicated to discovering therapies for rare neuroendocrine diseases previously untreatable or undertreated. Our science is focused on developing therapies for patients with hyperphagia (insatiable hunger) and severe obesity caused by an impaired melanocortin-4 receptor (MC4R) pathway disease. We are also investigating treatments for other rare diseases, including chronic hyperinsulinism.

Our areas of focus

We are focused on developing treatment options to restore the function of the MC4R pathway, including POMC, PCSK1, or LEPR deficiency; Bardet-Biedl syndrome, hypothalamic obesity, as well as congenital hyperinsulinism.

See more →

The MC4R pathway

We are focused on diseases caused by MC4R pathway impairments. These diseases manifest as hyperphagia and result from rare gene variants and/or injury to the hypothalamus, distinct from more common causes of obesity.

See the connection →

Research to treat severe obesity

Our approach to developing treatments is built upon our foundational DNA sequencing database.

Learn more →

Our pipeline and clinical trials program

We develop innovative therapies for rare diseases

Discover treatments →

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Rhythm Pharmaceuticals, Inc.
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Science

Understanding obesity and the MC4R pathway

"The MC4R pathway plays a pivotal role in the underlying biology of many forms of severe obesity and hyperphagia. We are committed to using our growing understanding of this pathway to develop new therapeutic options. . .to relieve the substantial burden of living with these rare diseases."

Dana W.
Senior Vice President, Clinical Development

Not all obesity has the same causes

We are focused on the severe forms of obesity caused by MC4R pathway impairments. These types of obesity result from rare gene variants and/or trauma to the hypothalamus, which are distinct from more common causes of obesity. These diseases are characterized by hyperphagia (insatiable hunger) and early- or rapid-onset, severe obesity.

~5M*

About five million* people in the United States (1.7% of the population) have early-onset, severe obesity

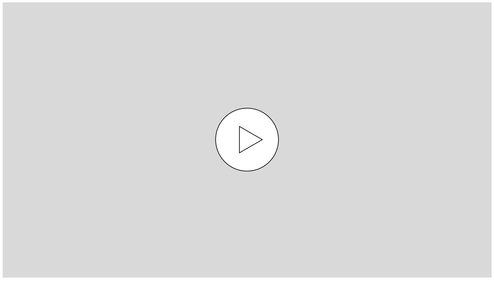
~95%

Roughly 95% of individuals with early-onset, severe obesity remain obese into adulthood

>53,000

More than 53,000 individuals in the United States with rare genetic obesities could respond to setmelanotide**

*Hales et al 2018; Ward et al 2017.
**Estimate based on sequencing-based epidemiology data and response rates to setmelanotide in clinical trials.



The MC4R pathway and severe obesity

All obesity is not the same.
The MC4R pathway is an endocrine pathway in the brain responsible for regulating hunger, caloric intake, and energy expenditure. When impaired, it can lead to rare genetic diseases characterized by severe obesity and hyperphagia.

IMCIVREE® (setmelanotide) activates the MC4R pathway by mimicking the effects of a naturally occurring molecule called MSH (melanocyte-stimulating hormone). This hormone plays a key role in regulating hunger and energy expenditure.

Learn more about the MC4R pathway and severe obesity

Rare Obesity

What is hyperphagia?

Hyperphagia is a pathological, insatiable hunger that has a significant impact on patients and their families.

Symptoms of hyperphagia may be extreme and constant. Hyperphagia is accompanied by abnormal food-seeking behaviors, which may include waking up at night to find food, eating food others leave behind, and in severe cases, sneaking, stealing or hiding food. Food seeking behaviors and symptoms of hyperphagia can be overwhelming and relentless, and hyperphagia brought on by impairment of the MC4R pathway leads to severe obesity.

Rhythm is uniquely focused on discovering new treatments that may offer hope and an improved quality of life for patients with hyperphagia brought on by rare genetic obesities and hypothalamic obesity, which is caused by brain trauma.

Discover more

Learn more about hyperphagia and severe obesity, how rare MC4R pathway diseases are diagnosed, and where to find help and support.

LEAD for Rare Obesity

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Science

Developing therapies to treat rare Neuroendocrine pathway diseases

We are focused on treating diseases that impact the MC4R pathway.

These include:

POMC/PCSK1/LEPR Deficiencies

Rare variants of the LEPR, POMC, and PCSK1 genes can result in deficiencies of certain proteins and enzymes that help the body regulate food intake, resulting in hyperphagia (insatiable hunger) and early onset obesity.

BBS (Bardet-Biedl syndrome)

A rare genetic disease that causes a variety of symptoms, including hyperphagia (insatiable hunger) and early-onset obesity, causing profound challenges for patients and their caregivers.

HO (Hypothalamic obesity)

This is caused by injury to the hypothalamus that disturbs the MC4R pathway, which regulates food intake, resulting in hyperphagia and rapid onset severe obesity.

Additional neuroendocrine focus area

Congenital Hyperinsulinism (CHI)

Congenital hyperinsulinism (CHI) is a rare condition that causes the body to produce too much insulin, resulting in persistent hypoglycemia (low blood sugar) in newborns and children that can cause seizures, coma, neurological damage, and even death.

Discover more

Learn more about these rare diseases

LEAD for Rare Obesity

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Science

Life-changing research to treat severe obesity and hyperphagia

Our research efforts are focused on translating the knowledge we gain from laboratory investigations and clinical studies to deepen our understanding of severe obesity and hyperphagia caused by rare melanocortin-4 receptor (MC4R) pathway-related diseases.

Our unique DNA Sequencing Data Platform

Our approach to understanding MC4R pathway diseases is built upon a DNA sequencing infrastructure that includes genetic samples from approximately 80,000 people with severe obesity.
(Each sample has been de-identified, or made anonymous, to protect the donor's privacy.)
This database, believed to be one of the largest of its kind, is a unique resource.

Genetic testing can play an important role in diagnosing many rare genetic diseases of obesity.
To help us understand the relevance of the variants we discover, we use tools like large-scale biochemical screening studies and genetic epidemiological analyses.

By deepening our understanding of the genetics that contribute to severe obesity, we aim to identify people living with rare MC4R pathway diseases who may benefit from targeted therapies.

Discover more

Learn more about our genetic testing program.

Uncovering Rare Obesity®

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Our pipeline:

Delivering innovative therapies for rare diseases

“Through multiple clinical trials, we are exploring opportunities to extend the reach of our precision medicine to address the needs of more patients with early-onset, severe obesity and hyperphagia. Our goal is to deliver new options to these patients, who otherwise have no approved therapies that specifically address the underlying cause of their rare MC4R pathway diseases.”

David M., MD
CEO

At Rhythm Pharmaceuticals, we are driven to deliver new treatments for people with rare diseases.

Through our clinical trial program, we are seeking to expand global access to our lead treatment, IMCIVREE® (setmelanotide), and have a meaningful impact on the lives of patients with rare MC4R pathway diseases and their families.

Discover more

Learn more about our clinical trials program

Clinical trials program

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Pipeline

Our clinical trial program: developing therapies for rare neuroendocrine diseases

These trials help us evaluate safety and efficacy for these potentially life-changing treatments.
We are committed to providing access to as many patients as possible through participation in our clinical trials.

If you think you may be eligible, speak with your healthcare provider about the possibility of participating in one of our clinical trials.

Setmelanotide daily | LB54640 | RM718 | Pre-Clinical Pipeline

PHASE 3: Hypothalamic Obesity Trial

- Patients aged 4 and older with acquired hypothalamic obesity

PHASE 3: EMANATE

- POMC insufficiency obesity
- LEPR insufficiency obesity
- SRC1 deficiency obesity
- SH2B1 deficiency obesity

PHASE 2: DAYBREAK

- Additional genes with strong or very strong relevance to the MC4R pathway

Discover more

Learn more about our trials at https://clinicaltrials.gov/

Learn more

Understanding Clinical Trials

Clinical trials are scientific studies where researchers evaluate a treatment, like a new drug, in human volunteers. In most cases, new medicines must pass through clinical trials to show their safety and effectiveness for approval from the US Food and Drug Administration (FDA), the European Medicines Agency (EMA), and other health regulators.

What You Should Know About Clinical Trials

- A patient must meet specific eligibility criteria to participate in a clinical trial, such as age, diagnosis, and treatment history
- There may be some risk in participating in a clinical trial, though participants' safety is closely monitored
- There is usually no cost to receiving treatment in a clinical trial
- Volunteers in a clinical trial may withdraw at any time
- For information about a clinical trial that could be a good fit for you or a loved one, talk to your doctor. Ask your doctor to email clinicaltrials@rhythmtx.com for more information

Expanded access to investigational drugs

Patients may be able to receive investigational therapies through the expanded access program (EAP) (sometimes called compassionate use), if:

- Patient is unable to participate in clinical trials, and
- Patient has a serious or immediately life-threatening disease or condition with no comparable or satisfactory alternative treatment options, and
- Potential benefit of treatment outweighs the risk

Following the completion of a Phase 3 clinical trial that meets key clinical endpoints, Rhythm may consider making setmelanotide available through our EAP. If a treating physician believes that setmelanotide may be the only therapeutic option for a patient along with the parameters listed above, they can request additional information about Rhythm's EAP by emailing: ExpandedAccess@rhythmtx.com. Rhythm will acknowledge receipt of such requests within five (5) business days.

Investigator-initiated Studies

Rhythm's areas of interest for investigator-initiated studies are focused on neuroendocrine diseases, including but not limited to natural history, genotype-phenotype correlations, energy expenditure, hyperphagia and real-world experience with setmelanotide. Studies designed for general obesity or non-rare diseases are not within our scope. For more information, please email iis@rhythmtx.com.

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Our commitment to patients and caregivers

We are defined by our ability to deliver rare impact. We develop medicines for previously untreatable or undertreated diseases and provide meaningful support for healthcare providers, patients, their families, and their communities.

Everyone at Rhythm knows that behind each discovery is a person whose life can be changed by our work. Our commitment to people living with rare neuroendocrine diseases extends well beyond the discovery of novel treatments.

“We truly support the patients across their journey, it's a commitment I've never seen before at any other company.”

Jamie P.

1/4

Discover more

Learn about our disease focus →

View patient resources →

Contact Us →

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Patients

Understanding rare MC4R pathway diseases

We are committed to discovering and developing treatments for rare MC4R pathway diseases. This pathway plays a critical role in how the brain regulates hunger and energy expenditure (calorie burning).

MC4R pathway function can be disturbed by certain gene variants or injury to the brain region known as the hypothalamus. This can cause **hyperphagia** (insatiable hunger) and **severe obesity**.

Here is a brief look at these rare diseases of the MC4R pathway.

Overview of rare MC4R pathway diseases

LEPR/PCSK1/POMC deficiencies

Biallelic variants in the LEPR, PCSK1 and POMC genes can impair the MC4R pathway. This can cause hyperphagia and early-onset, severe obesity

Bardet-Biedl Syndrome (BBS)

Bardet-Biedl syndrome (pronounced BAR-day BEED-el, or BBS) is a rare genetic syndrome resulting in a variety of clinical features that evolve over time, which may include hyperphagia and early-onset, severe obesity arising from impairment of the MC4R pathway

Hypothalamic Obesity (HO)

Hypothalamic obesity (HO) is an acquired injury to the hypothalamic region. It can happen during surgery to remove a brain tumor and may impair activation of the MC4R pathway. People with HO may often develop hyperphagia, decreased energy expenditure, and fast-onset severe obesity

Discover more

Learn more about these rare diseases

LEAD for Rare Obesity

Other neuroendocrine diseases

Congenital hyperinsulinism

Congenital hyperinsulinism (CHI) is a rare condition that causes the body to produce too much insulin. It results in persistent hypoglycemia (low blood sugar) in newborns and children and can cause seizures, coma, neurological damage, and even death.

We are doing research to better understand CHI and developing much-needed therapies to help parents control their children's blood sugar levels to protect them from the potentially harmful effects of hypoglycemia.

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Patients

We’re here to support patients and caregivers

Rhythm is deeply committed to ensuring that patients with rare neuroendocrine diseases and their caregivers receive the support and access they need through our extensive services and clinical trial program.

Uncovering Rare Obesity: free genetic testing

Genetic testing can help physicians diagnose some rare genetic obesities. However, it is often not covered by insurance, limiting patients’ ability to access the tests. To help expand access to genetic testing in the United States, we launched Uncovering Rare Obesity, a no-cost, comprehensive testing program for rare genetic diseases of obesity.

Our Uncovering Rare Obesity® gene panel includes seventy-nine genes and one chromosome region, which represent almost all of the most frequently tested genes associated with obesity. (Please note: This gene panel does not test for Prader-Willi syndrome.) Eligible individuals can receive a genetic test and two genetic counseling sessions (one before and one after the test) at no charge.*

*Covers cost of test only for eligible individuals; additional restrictions apply.

Get the details

Rhythm InTune: resources for patients and caregivers

Rhythm InTune is a support program for caregivers and people living with rare genetic diseases of obesity. The program provides resources, education, and information to fit the unique needs of individuals living with rare genetic diseases of obesity, including Bardet-Biedl syndrome, LEPR deficiency, PCSK1 deficiency, and POMC deficiency.

Learn more

Clinical trials

Our robust and active clinical trial program is constantly evaluating new treatment options for people with rare diseases that cause hyperphagia and severe obesity. Enrolling in a Rhythm clinical trial can give patients access to these potentially life-changing therapies.

Learn more

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The Rare Impact of Rhythm

Our Story

Rhythm was founded in 2008 in Boston by rare disease specialists.

Our first treatment, IMCIVREE® (setmelanotide), was licensed in 2010 and is the first and only FDA-approved and EC- and MHRA-authorized therapy that targets a root cause of hyperphagia and early-onset, severe obesity.

Rhythm became a publicly traded company in 2017. Now, we have grown to over 215 employees in North America and Europe. With a robust clinical trials program, we remain poised for even more growth in the near future. As we continue to grow, the one thing that will never change is our commitment to patients.

Our Mission

To transform the lives of patients and their families living with rare neuroendocrine diseases by rapidly advancing care and precision medicines that address the root cause.

Our Vision

Patients and families with rare neuro-endocrine diseases are able to live fuller lives and are transformed through optimal care.

It takes great people to make a great company

It's the people that make Rhythm so special.
Meet our leadership team and employees.

Our leadership is passionate about developing new therapies for patients.

Meet Rhythm's leadership team and Board of Directors →

Our employees strive every day to deliver rare impact.

Meet our people →

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About

Our Leadership

Rhythm is guided by strong leadership at every level. We share an unrelenting drive to develop new therapies for rare neuroendocrine diseases, offering hope and support to patients and their families.

Meet Rhythm's leadership team and Board of Directors

LEADERSHIP TEAM BOARD OF DIRECTORS

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and Chief Executive Officer

Jennifer Lee

Executive Vice President,
Head of North America

Yann Mazabraud

Executive Vice President,
Head of International

Hunter Smith

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Chief Technical Officer

Pam Cramer

Chief Human Resources Officer

Dana Washburn

Senior Vice President,
Clinical Development

Elisabeth Crönert-Bendell, MD, MBA

Senior Vice President,
Head of Strategy

Jim Flaherty

Senior Vice President
and General Counsel

Discover more

Learn more about our team and the people working at Rhythm.

Meet Our People

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LEADERSHIP TEAM BOARD OF DIRECTORS

David Meeker, MD

Chairman, President
and Chief Executive Officer

Dr. David Meeker, a member of Rhythm’s Board of Directors since 2015 and Chairman of the Board since 2017, was appointed President and Chief Executive Officer of the Company in July 2020. Most recently, he served as President and CEO of KSQ Therapeutics for approximately three years. Previously, David was the Executive Vice President and Head of Sanofi Genzyme, the specialty-care global business unit of Sanofi that focused on rare diseases, multiple sclerosis, oncology and immunology. He joined Genzyme in 1994 as Medical Director and, over the course of his tenure, served the company as Vice President of Medical Affairs, Chief Operating Officer, and Chief Executive Officer. He led Genzyme’s commercial organization and global market access functions and managed the launch of several treatments for rare genetic diseases, including Aldurazyme®, Fabrazyme® and Myozyme®. Prior to his tenure with Genzyme, David was Director of the Pulmonary Critical Care Fellowship at the Cleveland Clinic and an Assistant Professor of Medicine at Ohio State University. Dr. Meeker earned his M.D. from the University of Vermont Medical School and completed the advanced management program at Harvard Business School.

The heart of Rhythm is our people

At Rhythm, we believe in the work we are doing and are driven to help improve the lives of patients and families living with rare neuroendocrine diseases by advancing science, developing innovative therapies, and providing unparalleled support.

The Rhythm company culture emphasizes strong collaboration across departments, bringing together colleagues with diverse ideas and perspectives in a collegial atmosphere.

“I could not be prouder to tell people what I do. In my 30-year career this is the happiest I have ever been.”

Sarah R.

“We truly support the patients across their journey, it's a commitment I've never seen before at any other company.”

Jamie P.

Discover more

Learn about career opportunities at Rhythm

See current opportunities

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IMCIVREE® (setmelanotide) injection: the first treatment to target an impaired MC4R pathway

IMCIVREE® (setmelanotide)

IMCIVREE® (setmelanotide), is a precision medicine designed to treat hyperphagia (insatiable hunger) and severe obesity caused by an impaired melanocortin-4 receptor (MC4R) pathway.
IMCIVREE® is the first approved therapy for certain rare MC4R pathway diseases in the United States, Europe, Great Britain and several other countries.

In collaboration with leading experts across the world, Rhythm is advancing the most comprehensive clinical research program ever initiated in MC4R pathway diseases, including a Phase 3 trial for acquired hypothalamic obesity.

IMCIVREE® (setmelanotide) is approved for chronic weight management in adult and pediatric patients 6 years of age and older with obesity due to Bardet-Biedl Syndrome and LEPR, POMC, or PCSK1 deficiencies.
For more information, including important safety information, visit IMCIVREE.com.

See Full Prescribing Information here.

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Working at Rhythm

A fast-growing company with an exciting future

At Rhythm, we know our work makes an important difference in the lives of patients and their families around the world. Every employee contributes to the company's goal of discovering new treatments for rare diseases that no other company is developing.

As a fast-growing company with an exciting future, we offer a collaborative environment where employees' contributions truly matter.

"I truly see the impact Rhythm has on employees, communities, and most of all patients and their families. Rhythm is so much more than a job or a career...it's a place filled with greater purpose."

Pam C.

Meet Our People

Discover more

Contact us to apply for a job at Rhythm:
rhythmpharmaceuticals.applytojob.com/apply

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Contact Us

Thank you for your interest in Rhythm. If you would like to send us a question or comment, please fill out the form below.

Area of Inquiry*

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For Other Global Medical information medical inquiries:
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Search

Bardet-Biedl Syndrome

2 results found

Bardet-Biedl Syndrome (BBS) →

Bardet-Biedl syndrome (pronounced BAR-day BEED-el, or BBS) is a rare genetic syndrome resulting in a variety of clinical features that evolve over time, which may include hyperphagia and early-onset, severe obesity arising from impairment of the MC4R pathway

Rhythm InTune: resources for patients and caregivers

Rhythm InTune is a support program for caregivers and people living with rare genetic diseases of obesity. The program provides resources, education, and information to fit the unique needs of individuals living with rare genetic diseases of obesity, including **Bardet-Biedl syndrome**, LEPR deficiency, PCSK1 deficiency, and POMC deficiency.

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Search

Enter your search term

Popular searches:

MC4R

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Obesity

Quick links

Careers →

At Rhythm, we know our work makes an important difference in the lives of patients and their families around the world. Every employee contributes to the company's goal of discovering new treatments for rare diseases that no other company is developing.

Patients →

We are defined by our ability to deliver rare impact. We develop medicines for previously untreatable or undertreated diseases and provide meaningful support for healthcare providers, patients, their families, and their communities.

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Delivering rare impact for

Developing precision medicines for

LOGO

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