Seeking Gene Therapy Cures

Advancing an integrated pipeline of genetic therapies that correct the root cause of complex and rare life-threatening childhood disorders

Nasdaq: RCKT
June 2022

Year Founded	Locations in	# of Employees	Total Assets (12/31/21)	Cash and STIs (3/31/22)	Total Liabilities (12/31/21)
2015	**New York, NY Cranbury, NJ**	**~200**	**$497,020,000**	**$346,593,000**	**$42,296,000**

Rocket Pharma is a fully integrated, clinical-stage company advancing gene therapies with curative potential for multiple rare childhood diseases.

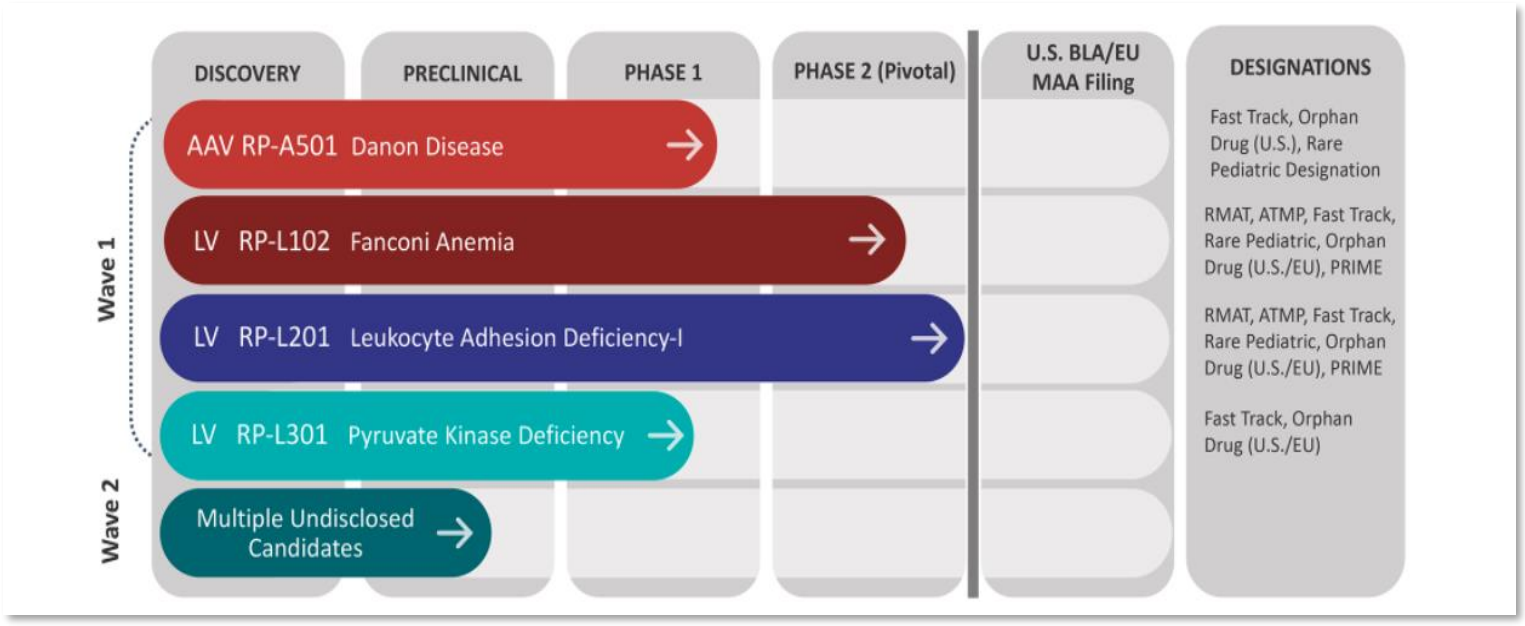
Rocket's Science

Rocket targets disorders driven by mutations in a single gene and develops therapies that directly address these mutations in the affected cells and tissues. The company is platform agnostic and chooses each program's gene therapy approach based on what is most practical for the disorder being targeted. Each program is intended to be transformative, enabling not only a reversal of the disorder at molecular and cellular levels, but also sustained relief from debilitating and potentially life-threatening symptoms. This patient- and disease-focused approach, combined with a state-of-the-art, adeno-associated viral vector (AAV) manufacturing facility in Cranbury, New Jersey with expanded capabilities to support commercial production, position Rocket to meet patients' unmet medical needs across multiple rare and life-threatening diseases.

Rocket offers a sustainable pipeline of therapies with potential to transcend current treatment options for these rare disorders. Unique to Rocket, this pipeline is comprised of first-in-class gene therapies that utilize both adeno-associated viral vector (AAV) and lentiviral vector (LVV) approaches to gene therapy.

Science and data drive all product development decisions to address patients' unmet medical needs. Additionally, the first-in-class nature of each program enable Rocket to make independent, thoughtful decisions based on optimal clinical and scientific considerations.

With this foundation, Rocket is ideally positioned to launch a sustainable pipeline of therapies that will meaningfully expand current treatment options for these rare and devastating disorders.



Patients and Community

Patients with rare diseases are Rocket's North Star. They are the company's inspiration and purpose. Understanding their journey provides our team the guidance to seek gene therapy cures.

Living with a rare disease is an ongoing and often relentless challenge for patients and families. In many cases there is no cure and there are frequent setbacks and frustrations. Living with a rare disease involves fighting misconceptions, finding informed and compassionate caregivers and seeking accurate information about available treatment options. A particularly challenging aspect is that rare disease patients do not have the luxury of time. This inspires Rocket to be laser focused on developing first-and-only-in-class therapies in the most efficient manner possible.

Rocket researches mechanism by which healthy genes can be inserted into affected cells to enable biologic correction. Some of the diseases currently under study include:

Fanconi Anemia (FA)	Leukocyte Adhesion Deficiency-I (LAD-I)	Danon Disease (DD)	Pyruvate Kinase Deficiency (PKD)

Mission and Sustainability

Rocket's core values and sustainability practices are closely aligned with the Biopharma Investor ESG Communications Guidance 2.0, as developed by the Biopharma Sustainability Roundtable. Several high-priority ESG topics are integrated with Rocket's overall strategic communications strategy:

1. The roles of Chairman (Rod Wong) and CEO (Gaurav Shah) are separate.
2. Compensation, including equity compensation, is based on a combination of personal and corporate performance. As disclosed in Rocket's proxy statement, the corporate attainment this year was 90% of goals, and bonuses and equity awards reflect that attainment.
3. Patient safety is a paramount concern; it is assessed on an ongoing basis as part of the company's clinical trial protocols.
4. In accordance with Title 12 of the Code of Federal Regulations, 12 CFR 1710.19, Rocket has an Enterprise-Wide Risk Management program in place, which reports into the Audit Committee on regular intervals and **assesses** and mitigates risk across functions organization-wide.

Rocket's new 103.000 sq. ft. adeno-associated virus cGMP manufacturing facility in Cranbury, N.J.

Leadership

The Rocket team possesses decades of experience and leadership in the biotech industry, with a combined total of more than 20 product approvals in the United States. This expertise has enabled the company to grow at an unprecedented rate and activate four clinical programs over recent years, including two in pivotal/registrational stage.

Kinnari Patel, PharmD, MBA
President & Chief Operating Officer

Carlos Martin
Chief Commercial Officer, SVP

Gayatri R. Rao, MD, JD
Chief Development Officer of LVV, SVP

Gaurav Shah, MD
Chief Executive Officer

Raj Prabhakar, MBA
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