



LIFE 2020 REAL ESTATE
DEVELOPMENT SYMPOSIUM
SCIENCES



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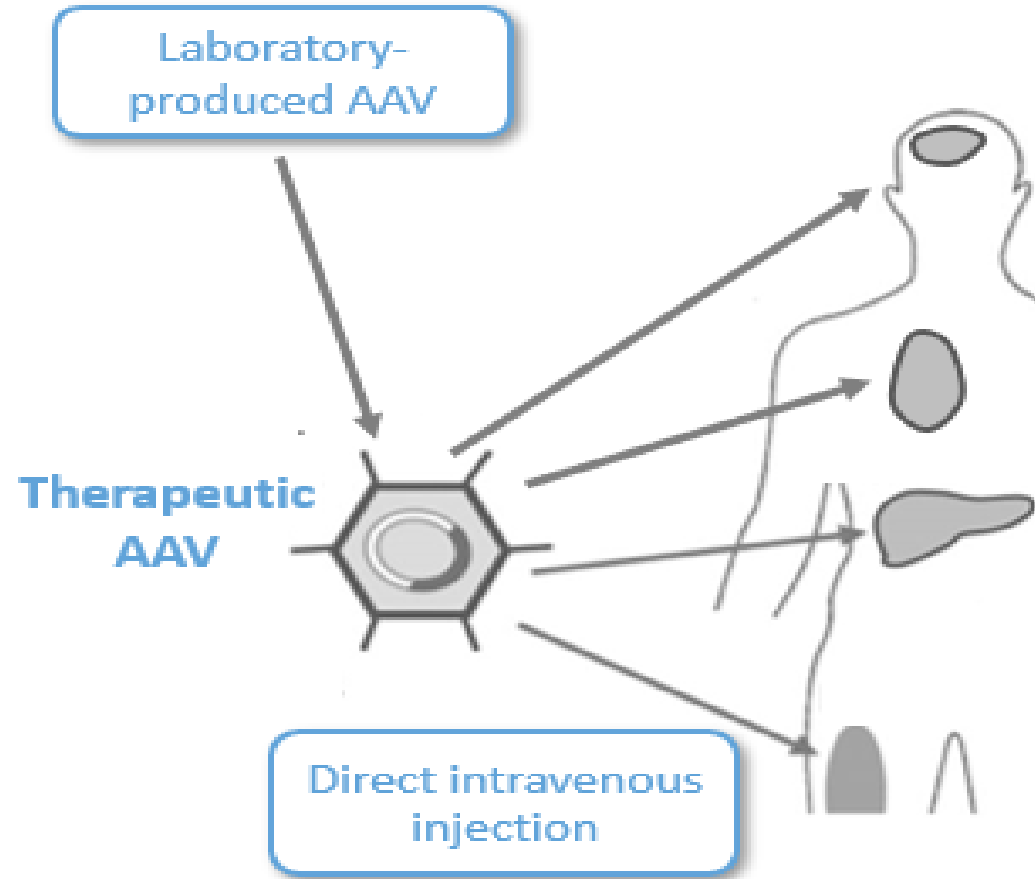
rocket
pharma

Gaurav Shah, MD
CEO & President

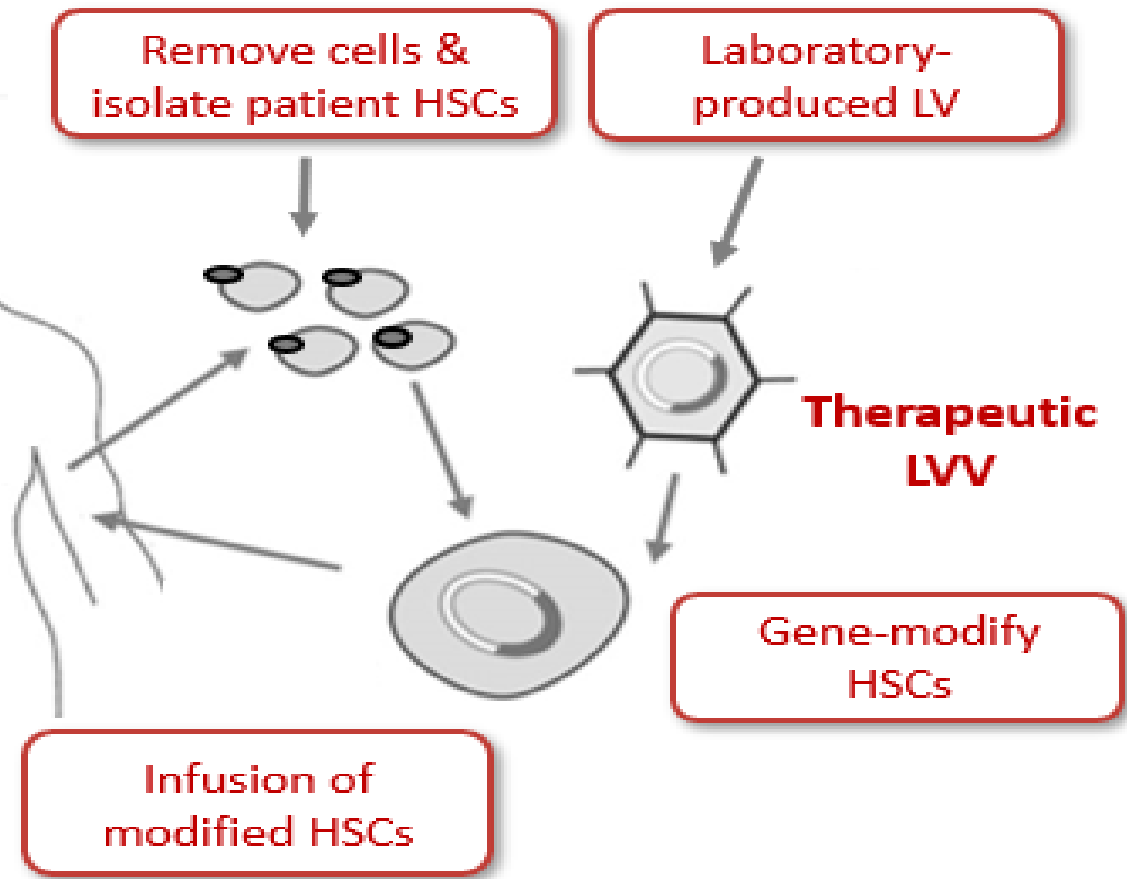


Gene Therapy: A Multi-Platform Approach

In Vivo (In Body) AAV Gene Therapy



Ex Vivo (Outside Body) Lentiviral Gene Therapy



About Rocket Pharma

Multi-Platform Gene Therapy (GTx) Company Targeting Rare Diseases 1st-in-class with direct on-target mechanism of action (MOA) and clear clinical endpoints

Ex-vivo Lentiviral vectors (LVV)

- Fanconi Anemia (FA)
- Leukocyte Adhesion Deficiency-I (LAD-I)
- Pyruvate Kinase Deficiency (PKD)
- Infantile Malignant Osteopetrosis (IMO)

In-vivo adeno-associated virus (AAV)

- Danon Disease

Multiple Near- & Medium-term Company Value Drivers

Near-term Milestones (2020)

- All five programs in the clinic (initiation of IMO)
- New preliminary data in Danon & PKD; Additional mature data in FA & LAD-I
- Two programs in registration-enabling Phase 2 (FA, LAD-I)

Medium-term Milestones (2021-2022)

- First global submission (BLA/MAA)
- Platform establishment and pipeline expansion
- Current programs eligible for Pediatric Priority Review Vouchers

Strong Precedents & World-Class Expertise

Strong Precedents and Sound Strategy

- Precedents for LVV- & AAV-based therapies
- Clearly-defined product metrics across indications
- Experienced company leadership
- Leading research and manufacturing partners



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