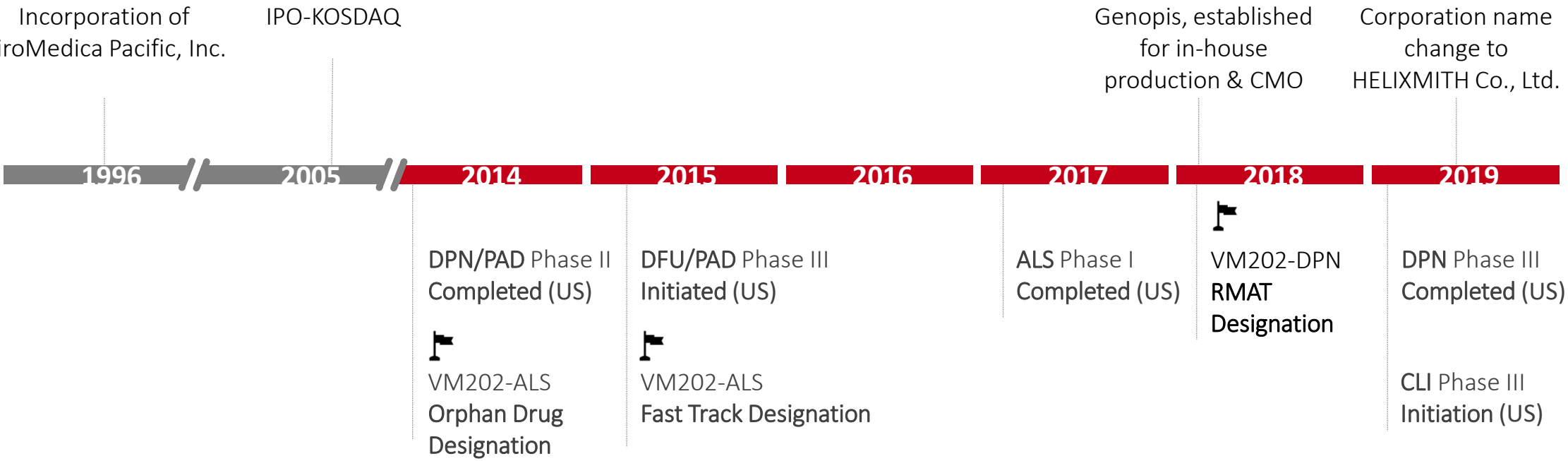
Helixmith Co., Ltd.

Nov 8, 2019

Helixmith History



Note: ALS=Amyotrophic Lateral Sclerosis; CAD=Coronary Artery Disease; CLI=Critical Limb Ischemia; DPN=Diabetic Peripheral Neuropathy; DFU=Diabetic Foot Ulcer;

Helixmith Overview

Pioneer and global leader in **plasmid DNA based gene therapy development** conducting multiple **late-stage clinical trials**, with a particular emphasis on diseases associated with neurological, muscular or ischemic problems

Headquarters and R&D
Seoul, Korea

Headquarters and R&D (Dec. 2019)
Seoul, Korea

DNA Production Facility
San Diego, CA, USA

KOSDAQ
(084990)

HQ

R&D
oriented

110+
employees

Clinical Development

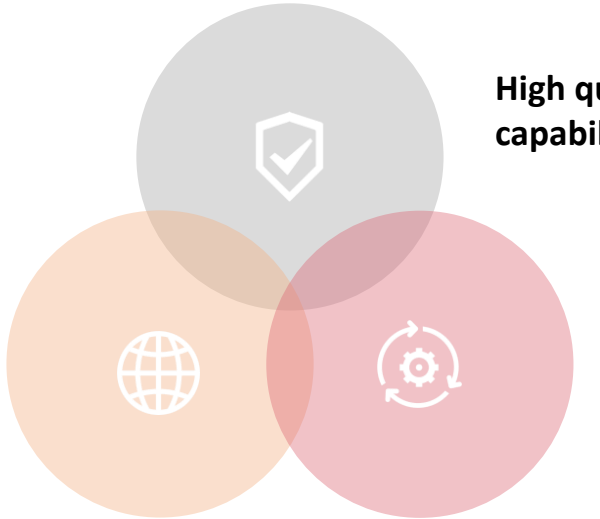
Production

40+
employees

Our Manufacturing Facility

Helixmith has established manufacturing facility in San Diego to solve the manufacturing bottleneck in biopharma industry with accumulated experiences and know-hows in gene therapy market.

Pioneer of commercial plasmid DNA manufacturing facility



High quality and reliable in-house production capability for both clinical and commercial scale

Contract Manufacturing Organization (CMO) service for other biopharmaceutical companies

Plasmid DNA Production Facility Specification

- GMP-ready production facility with successful experience in regulatory due diligence
- 68,400 ft^2 plant
- 500 L fermenter, cell culture lab and QC test lab, etc.
- Extra space (> 174,000 ft^2)to be equipped with 60-300L and 5-50L fermenter
- 40+ employees highly experienced in large-scale production of plasmid DNA

Our Team

Sunyoung Kim, DPhil

CEO

- Founder and CEO of Helixmith Co., Ltd.
- Professor, Seoul National University
- Assistant Professor, Harvard Medical School
- D.Phil.(Molecular Genetics), University of Oxford
- MS(Biochemical Engineering), MIT
- MA(Microbiology), Harvard University

William Schmidt, PhD

Clinical Operations

- DuPont Pharmaceuticals, Inc.
- Adolor Corporation
- Limerick Biopharma, Inc.
- Ph.D.(Pharmacology), UCSF

Keith Hall, MBA

COO

- Vical Inc.
- Amgen Inc.
- Hybritech Inc.
- MBA, University of Houston

Cindy Fisher, PhD

Regulatory Affairs

- Vical Inc.
  (pDNA based vaccine and novel antifungal)
- Ph.D.(Physical Organic Chemistry), UC Irvine

Gary Neumann

Head of Quality

- Serpta Therapeutics Inc.
- Ipsen/Tercica
- Novartis/Chiron Corp.
- Genentech, Inc.

Seungshin Yu, PhD

Head of Biologics

- Takara Bio Inc., Japan
- ViroMed Co., Ltd.
- Ph.D.(Virology), Seoul National University

Michael Na, CPA

CFO

- Nomura Financial Investment
- Macquarie Securities
- Deloitte & Touche LLP
- MS(Accounting), Ohio State University

Our Major Therapeutic Platforms

As a pioneer of gene therapy, Helixmith has been developing on the following therapeutic platforms with the promising technical know-hows, which will be a viable approach to treat human diseases

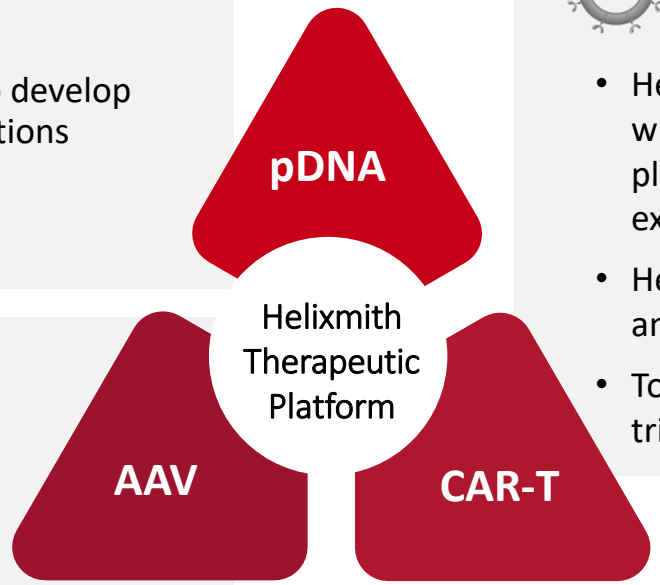
Gene Therapy

Plasmid DNA

- Helixmith has promising technical know-hows to develop gene therapies targeting a broad range of indications
- Hepatocyte Growth Factor (HGF)
- Insulin-like Growth Factor1 (IGF-1)

Adeno-associated Virus (AAV)

- Through animal studies, Helixmith discovered the most effective method for viral vector delivery – intrathecal injection

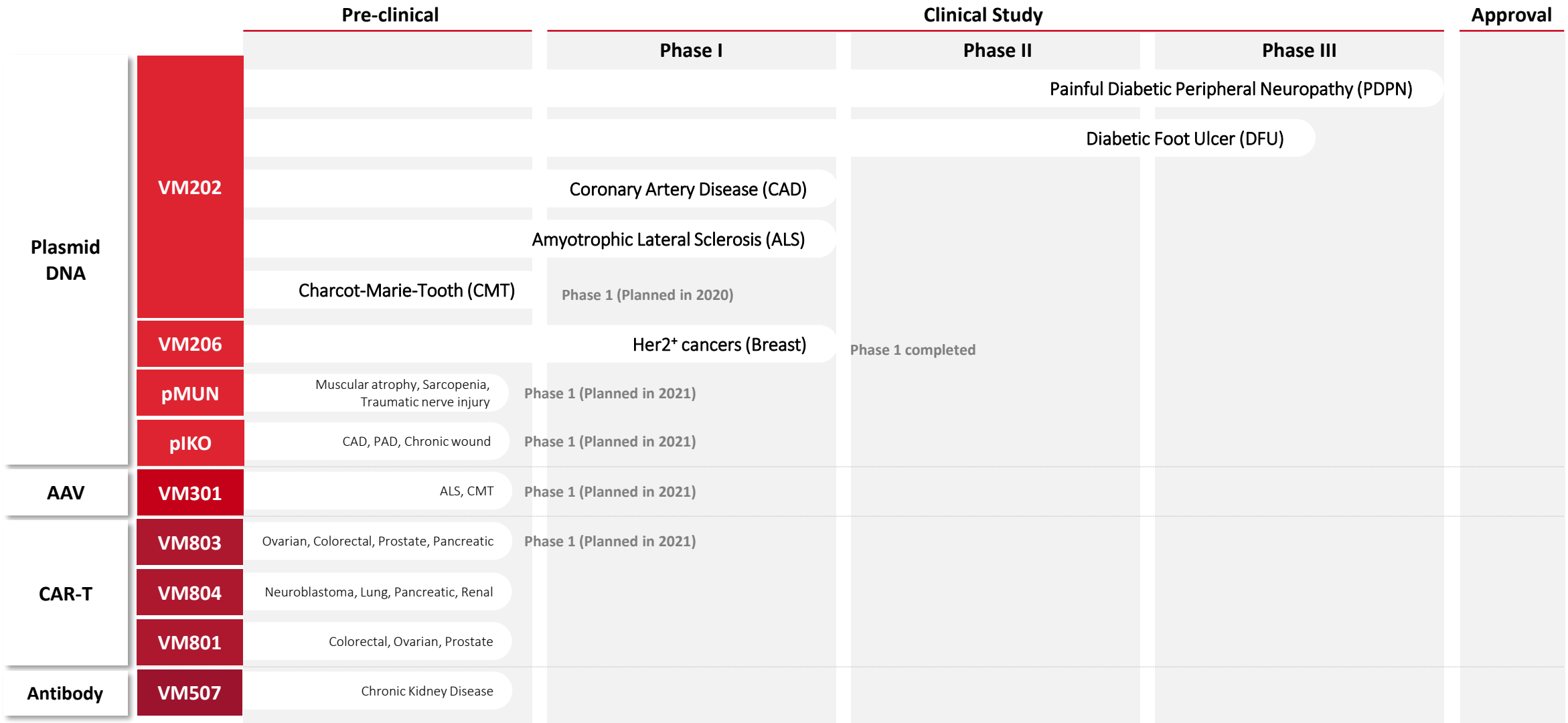


Cell and Gene Therapy

CAR-T

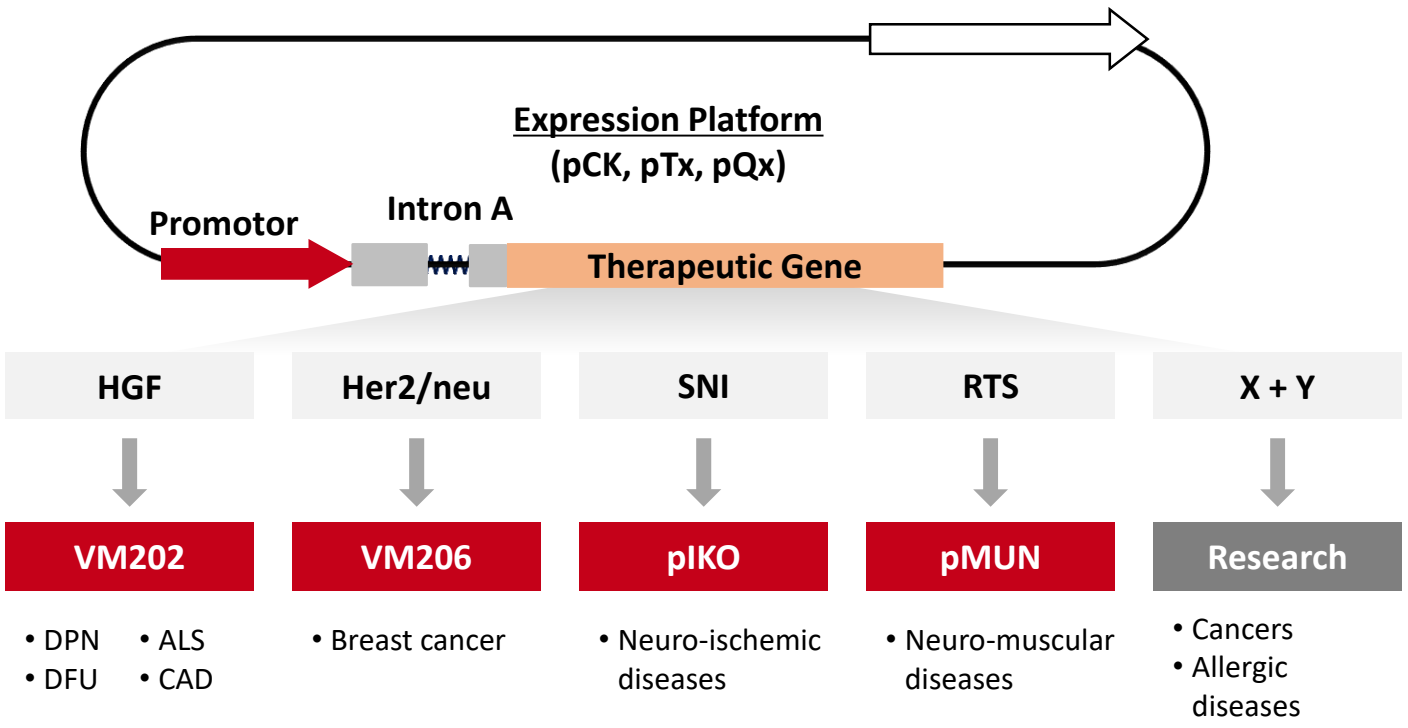
- Helixmith has been developing CAR-T therapies with its exclusive retroviral gene delivery platform, optimized for safety and gene expression efficiency
- Helixmith is developing CAR-T with its unique antibody structure targeting solid tumors
- Top four leading pipelines are aiming for clinical trials in 2022

Helixmith Portfolio



Our Robust Platform Technology

Helixmith has a robust plasmid DNA platform technology to develop **best-in-class drugs with breakthrough transgene expression level** by inserting different types of therapeutic genes to the expression platform



Breakthrough transgene expression level

- IP-protected Intron A cassette drives best-in-class gene expression
- Ideal for localized expression without increase in other tissues or serum

Best Safety profile

- Suitable for diverse indications
- No detectable risk of genomic integration and oncogenesis

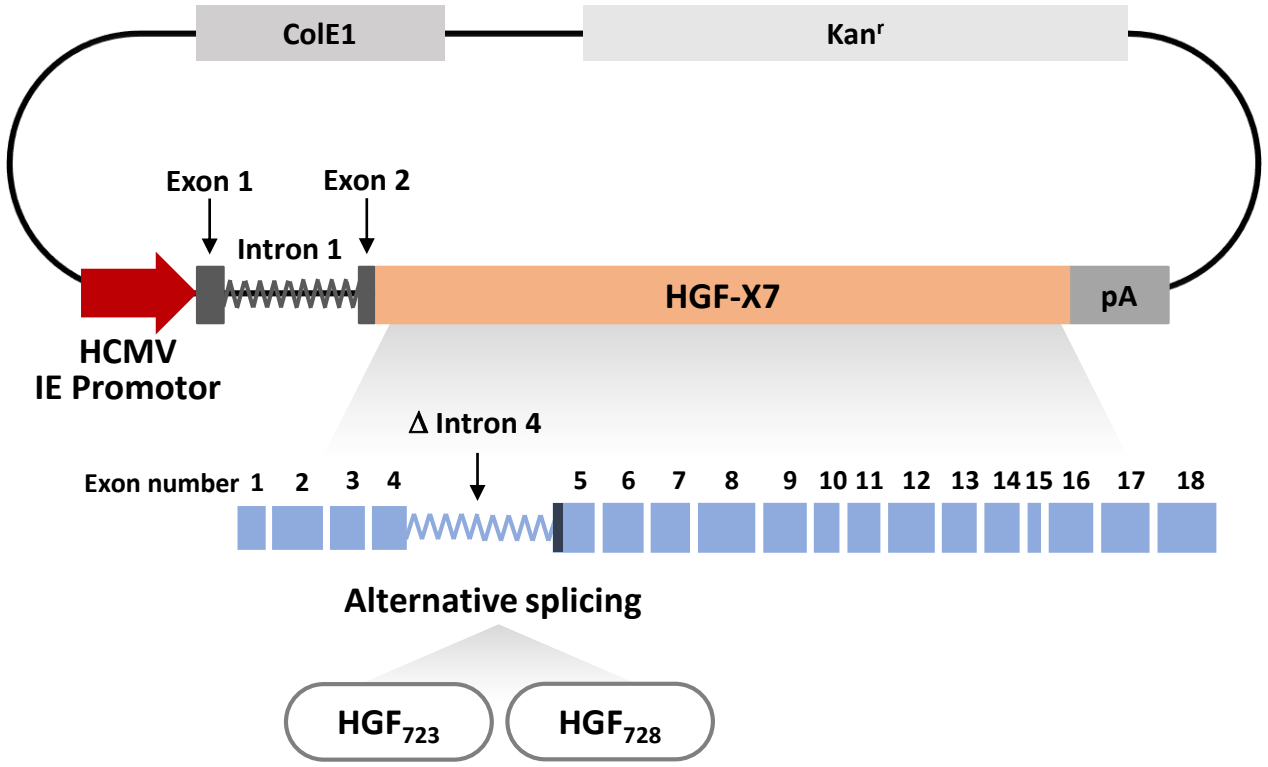
Streamlines production process

- Proprietary formulation
- Simpler process and lower manufacturing cost compared to other modalities

Note: DPN=Diabetic Peripheral Neuropathy; DFU=Diabetic Foot Ulcer; ALS=Amyotrophic Lateral Sclerosis; CAD=Coronary Artery Disease

Flagship Product VM202 Engensis®

VM202 is a novel genomic cDNA hybrid HGF gene with a novel and proprietary coding sequence, HGF-X7, expressing two isoforms needed for optimal therapeutic benefits



1. **Construction of HCMV-based expression vector**
2. **Genomic-cDNA hybrid HGF, HGF-X7**
3. **High levels of gene expression**
4. **Maintained in high level for a long period**
5. **Excellent Safety**

Engensis® Clinical Trial

Target Indications of VM202 under clinical studies

Amyotrophic Lateral Sclerosis

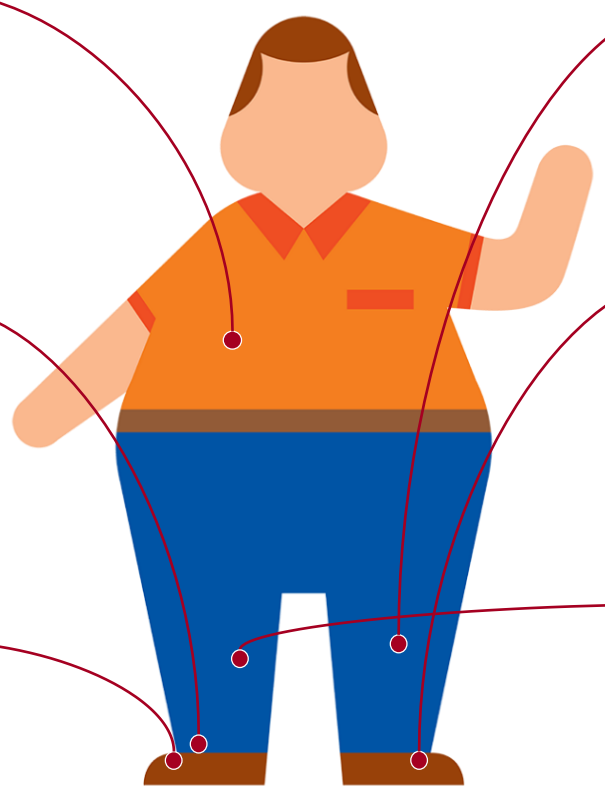
Phase II
Planned in US (2019)

Painful Diabetic Peripheral Neuropathy

Phase III
Ongoing in US

PDPN Opioid Sparing study

Phase II
Planned in US



Claudication

Phase II
Ongoing in US

Diabetic Foot Ulcer (DFU)

Phase III
Ongoing in US

Charcot-Marie-Tooth disease

Phase I/II
Planned in KR and US (2020)

Engensis® Phase II and III (3-1B)

Engensis showed an excellent safety profile with significant improvements in all pain measures in patients with painful diabetic peripheral neuropathy through Phase II [n=102] and Phase III (3-1B) [n=101].

Excellent safety profile with no major drug-related AEs or SAEs

Significant efficacy in pain reduction for a long period of time (6 to 12 months; Daily pain diary, BPI-DPN, VAS, PGIC)

Much greater pain reduction observed in the patients not on *pregabalin* and/or *gabapentin*

Potential to be regenerative medicine
Sustained pain reduction even after the disappearance of VM202 and HGF protein

Engensis® Upcoming Plans

1 **Conduct 2 to 3 mid-sized Phase III trials**

- 150 to 200 subjects per trial
- 5 to 7 sites per trial
- Assure optimal data quality on each site by employing state of art methodology

2 **Target optimum efficacy population**

- Primary endpoint: Daily Pain Diary at 6 months
- Recruit subjects not on gabapentin/pregabalin

3 **Demonstrate regeneration capacity through long-term clinical trial**

- Conduct roll-over extension study with the subjects from above Phase III studies
- Assess nerve regeneration from above Phase III studies after 15 years