

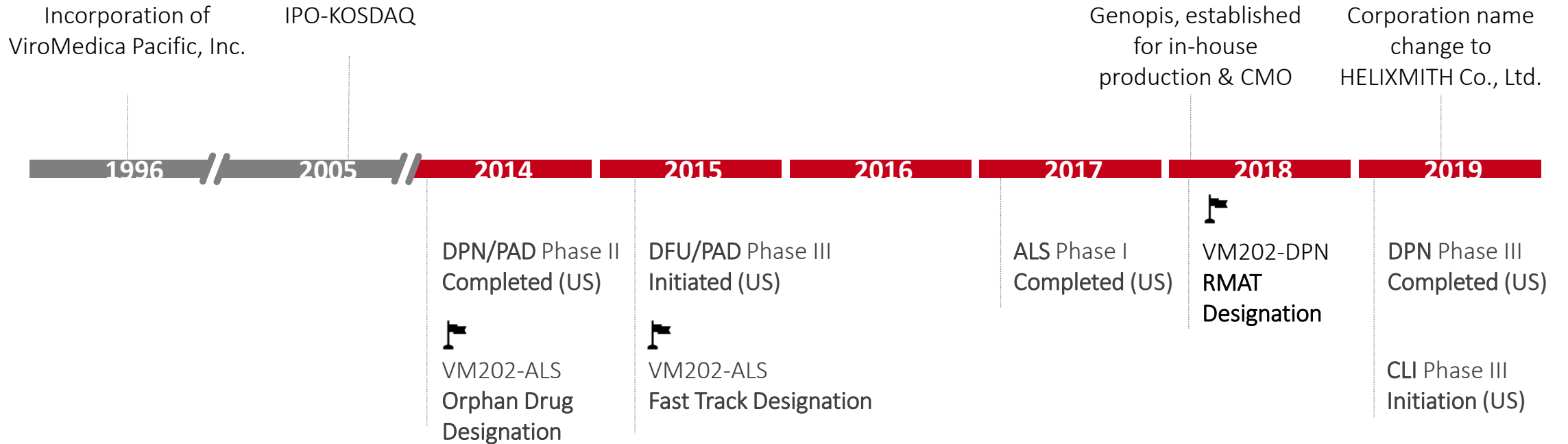


HELIXMITH

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



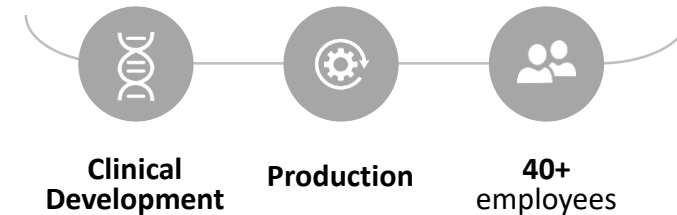
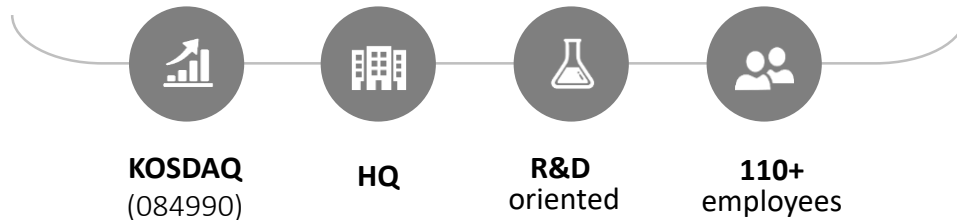
Headquarters and R&D
Seoul, Korea



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

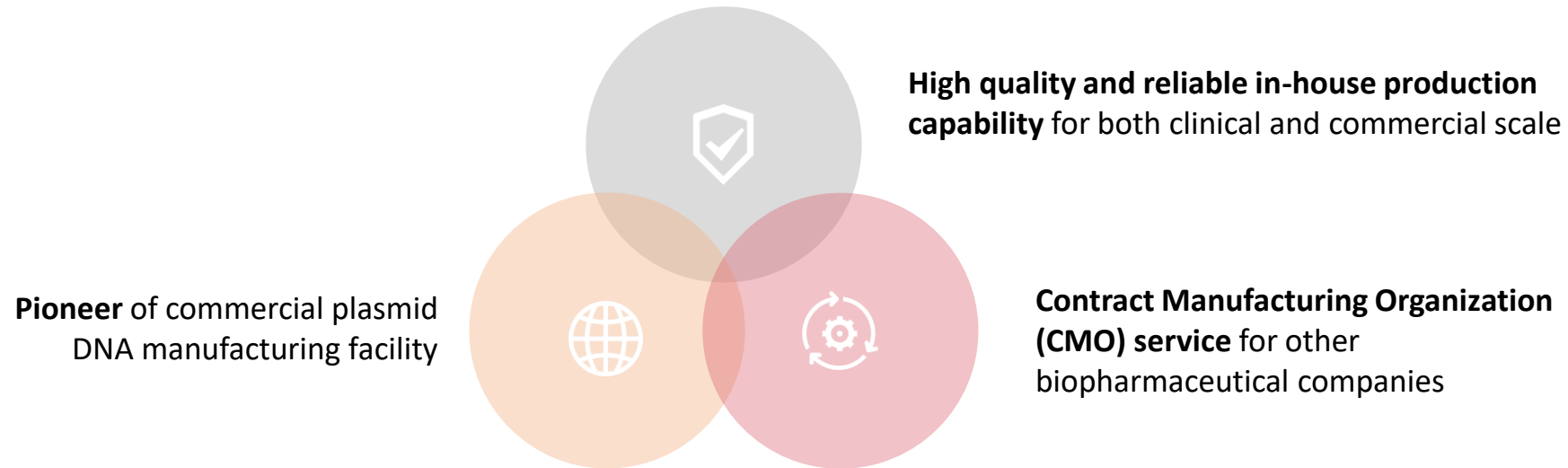


DNA Production Facility
San Diego, CA, USA



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.



Plasmid DNA Production Facility Specification

- GMP-ready production facility with successful experience in regulatory due diligence
- 68,400 ft² plant
- 500 L fermenter, cell culture lab and QC test lab, etc.
- Extra space (> 174,000 ft²) 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



Cindy Fisher, PhD
Regulatory Affairs

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



Seungshin Yu, PhD
Head of Biologics

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



Keith Hall, MBA
COO

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



Gary Neumann
Head of Quality

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



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)

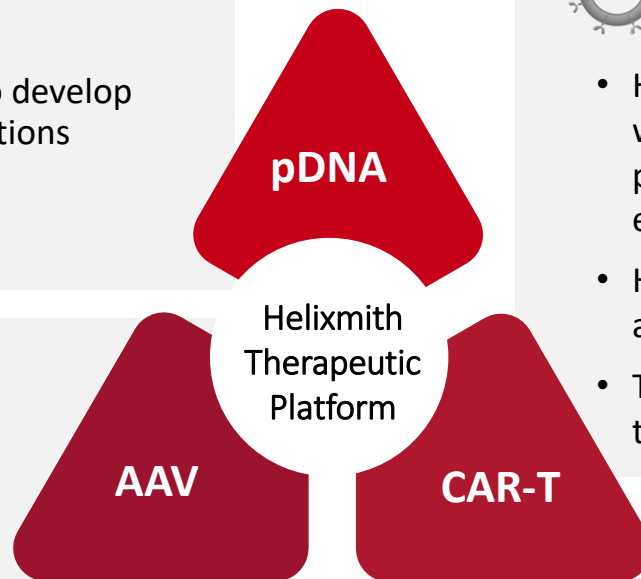
- Through animal studies, Helixmith discovered the most effective method for viral vector delivery – intrathecal injection

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

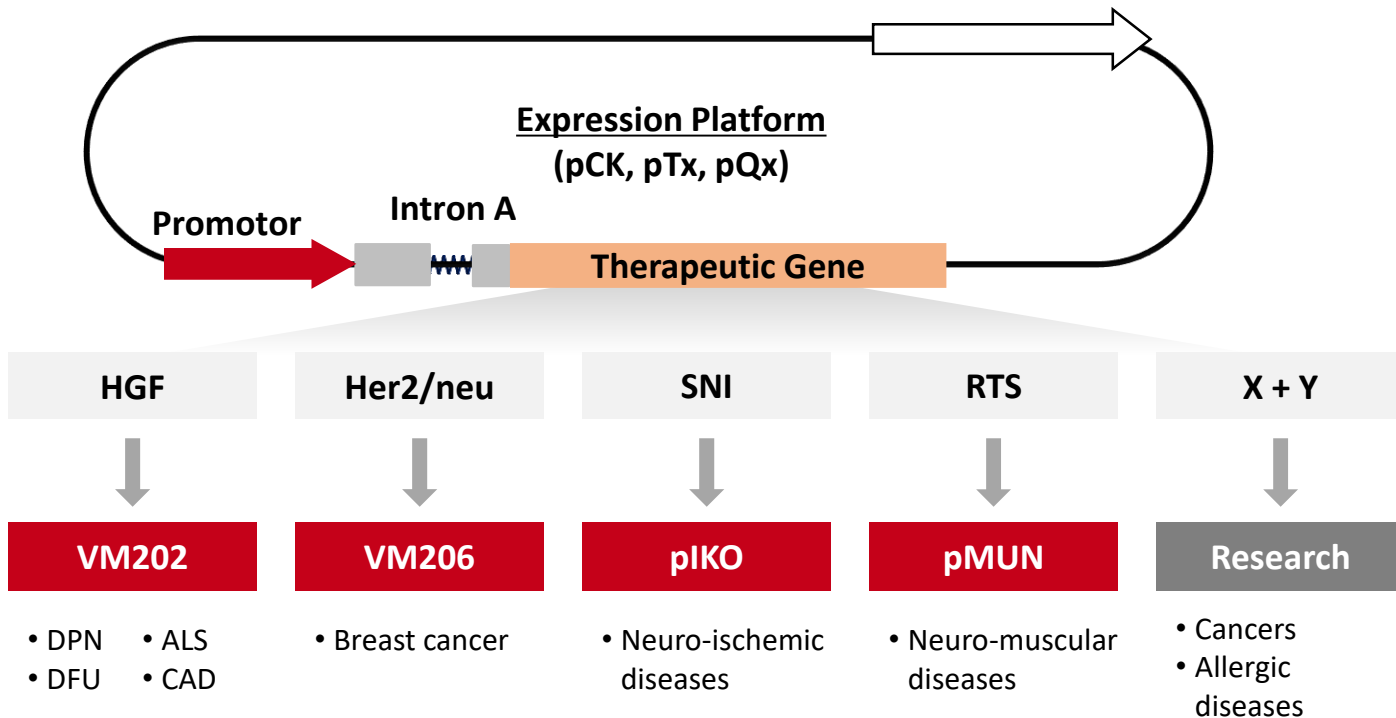


Helixmith Portfolio

		Pre-clinical	Clinical Study			Approval
			Phase I	Phase II	Phase III	
Plasmid DNA	VM202			Painful Diabetic Peripheral Neuropathy (PDPN)		
				Diabetic Foot Ulcer (DFU)		
			Coronary Artery Disease (CAD)			
			Amyotrophic Lateral Sclerosis (ALS)			
		Charcot-Marie-Tooth (CMT)	Phase 1 (Planned in 2020)			
	VM206		Her2 ⁺ cancers (Breast)	Phase 1 completed		
	pMUN	Muscular atrophy, Sarcopenia, Traumatic nerve injury	Phase 1 (Planned in 2021)			
	pIKO	CAD, PAD, Chronic wound	Phase 1 (Planned in 2021)			
AAV	VM301	ALS, CMT	Phase 1 (Planned in 2021)			
CAR-T	VM803	Ovarian, Colorectal, Prostate, Pancreatic	Phase 1 (Planned in 2021)			
	VM804	Neuroblastoma, Lung, Pancreatic, Renal				
	VM801	Colorectal, Ovarian, Prostate				
Antibody	VM507	Chronic Kidney Disease				

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

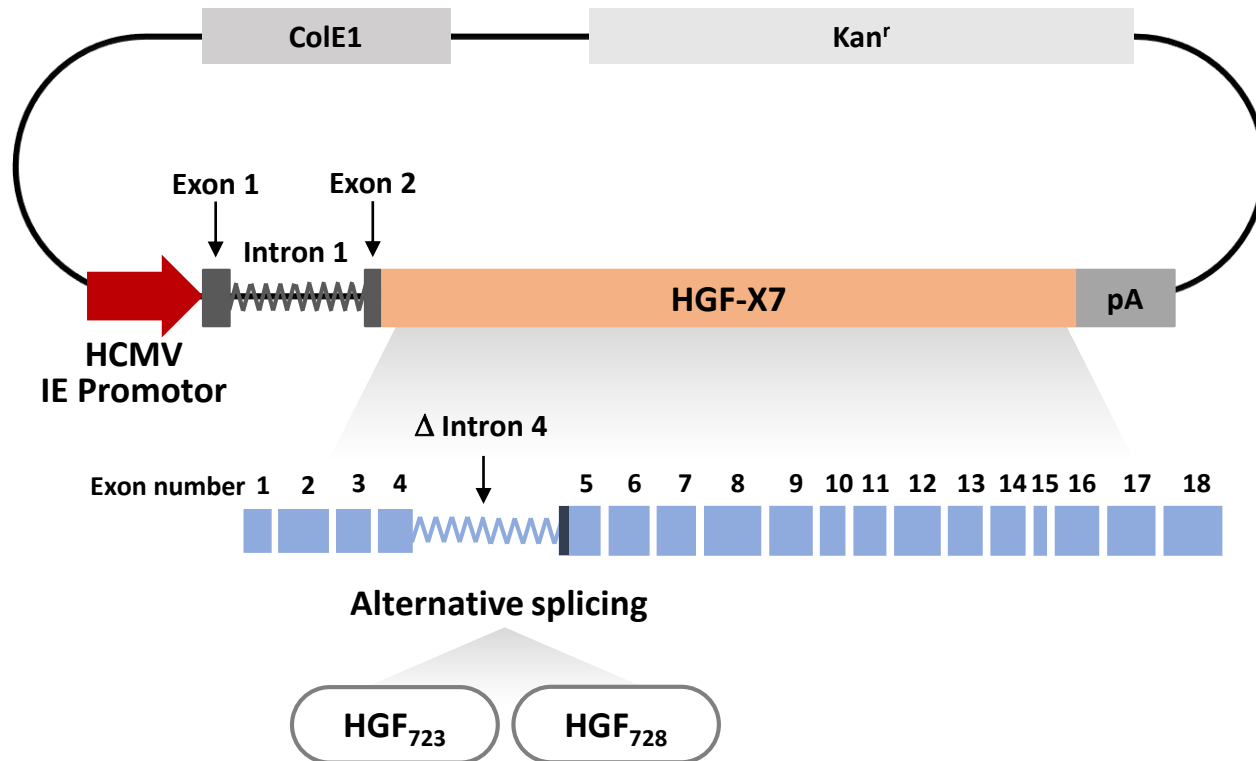


- Streamlines production process**
- Proprietary formulation
 - Simpler process and lower manufacturing cost compared to other modalities

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

Flagship Product VM202 Engensis®

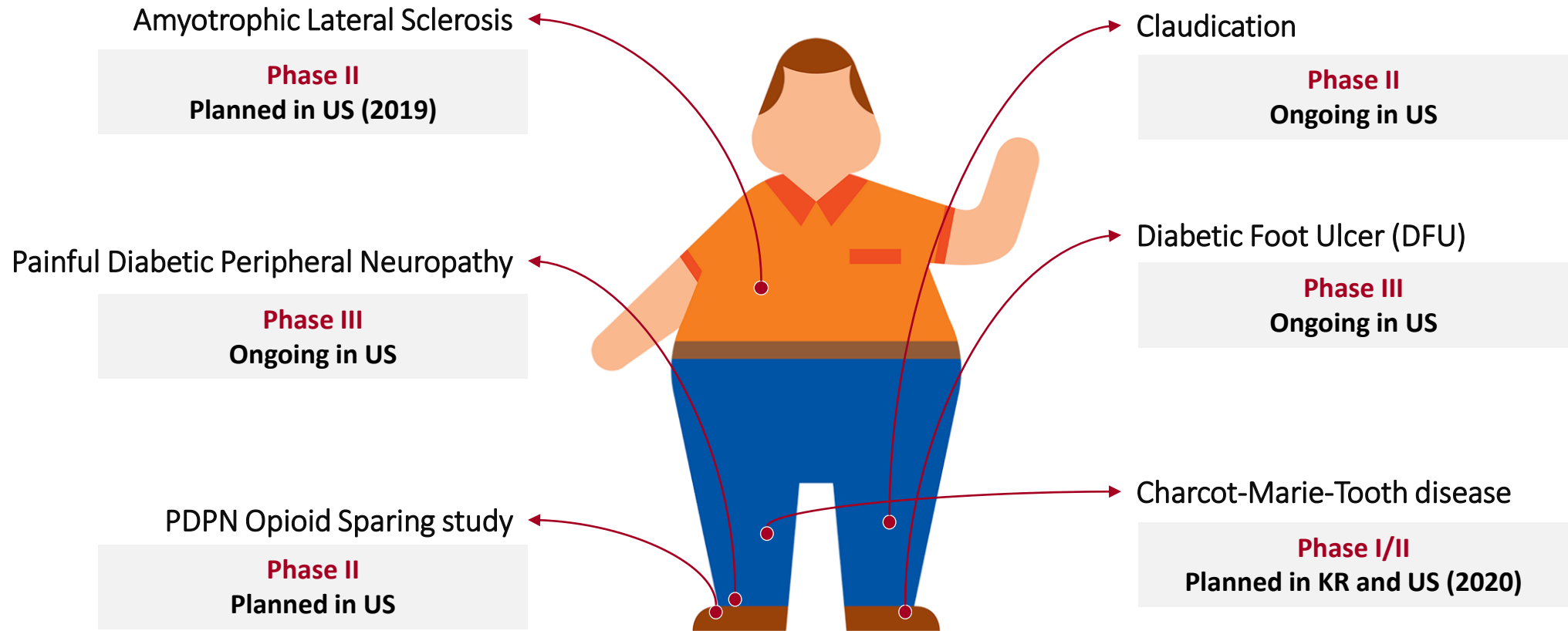
VM202 is a novel genomic cDNA hybrid HGF gene with a novel and proprietary coding sequence, HGF-X7, expressing two isoforms needed for optimal therapeutic benefits



- 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



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

이 종목의 더 많은 IR정보 [확인하기](#)

IR GO 주주와 기업을 연결하고 응원합니다.