Pioneering unique gene therapy approaches to provide
a life-long cure for patients suffering from genetic diseases

SANAGEN

SanaGen at a glance: pioneering unique gene therapy approaches to provide a life-long cure for patients suffering from rare genetic diseases

Gene therapy is the most promising approach to cure the cause of many diseases with a one-shot therapy in an effective and affordable manner.

We develop gene therapy candidates for rare genetic disorders originating from the liver by combining our unique, proprietary gene therapy innovations with off-the-shelf components.

Our convincing preclinical results, including PoC data for factor VII deficiency, endorse us to move towards clinical trials to treat the first patients with our lead gene therapy candidate.

An €8.0 million investment will enable us to advance our lead gene therapy candidate up to a Phase 1/2 clinical trial and optimize our gene therapy platform for other liver-related diseases.

Our team possesses all expertise and competencies to make SanaGen a succes

David Mosmuller, MD, PhD
Chief Executive Officer & Co-founder

Matthew Hotchko, PhD, MBA
Chief Business Officer & Co-founder

Bas Blits, PhD
Principal Scientist

We can rely on the support of relevant stakeholders and key partners

Sanquin

Dutch not-for-profit organization, charged with meeting healthcare's demand for blood and blood-related products, where leading medical, diagnostic and scientific knowledge about blood comes together.

NETHERLANDS INSTITUTE FOR NEUROSCIENCE
Master the mind

The NIN houses top researchers in neuroscience that perform research (fundamental and translational) into the functioning of the brain which in the longer term will lead to new treatments.

We have good relationships and contacts with many researchers and clinicians in the bleeding disorder community who have offered to be a site for clinical trials and late-stage development for our future gene therapy candidates.

Current treatments for diseases with genetic causes are not able to solve the cause and thereby cure the disease

To truly cure a disease, it is inevitable that the root cause of the disease needs to be tackled

40% OF ALL DISEASES HAS A GENETIC ROOT CAUSE

80% OF ALL RARE DISEASES IS OF GENETIC ORIGIN

For genetic diseases, this root cause is a defective, missing or mutated gene

If a therapy is even available, they have various shortcomings that impede optimal treatment

95% OF RARE GENETIC DISEASES HAS NO TREATMENT OPTION

"THERE IS A STRONG NEED FOR TREATMENTS THAT CAN SOLVE THE ROOT CAUSE OF GENETIC DISEASES, AND THEREBY PROVIDE A CURE TO ALL PATIENTS IN NEED"

Gene therapy is the approach to cure the cause of disease with a one-shot therapy instead of chronic, lifelong therapy to suppress the clinical consequences of a disease

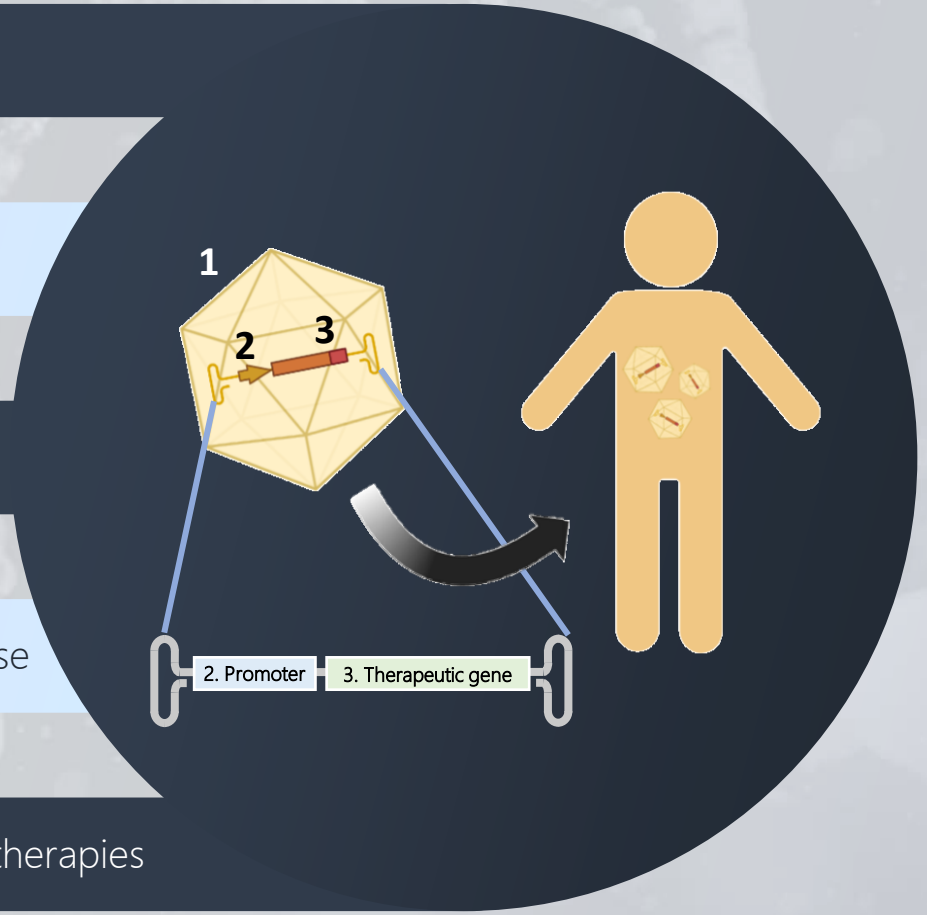
The purpose of gene therapy is to provide patients with functional genes

Gene therapy enables the patient's body to produce its own therapy

Gene therapies consist of a capsid, promoter and therapeutic gene

Gene therapy is a one-time treatment addressing the root cause of disease

Gene therapy can solve the efficacy and affordability problem of current therapies



SANAGEN

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GENE THERAPY IS THE ONLY TYPE OF THERAPY WITH THE GREAT PROMISE OF TRULY CURING PATIENTS

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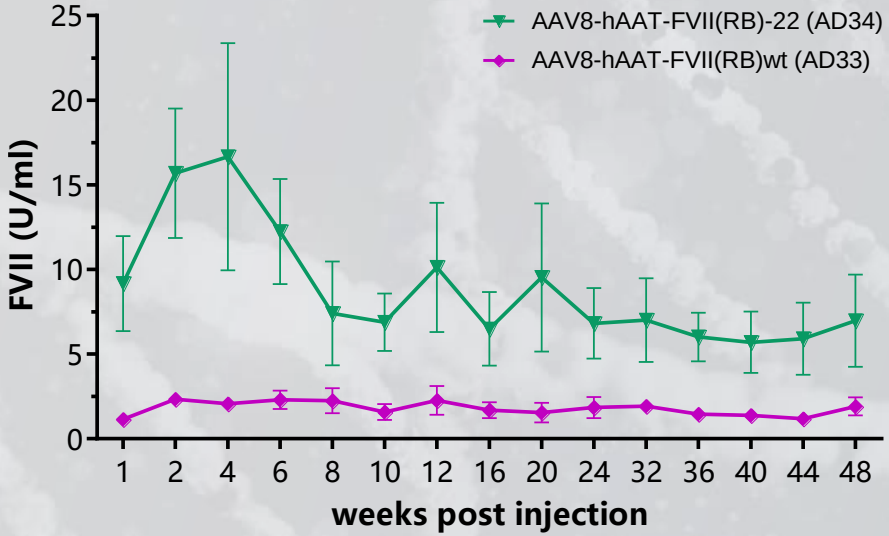
Our unique, proprietary gene therapy platform and approach has proven itself by establishing convincing preclinical results, including PoC for factor VII deficiency

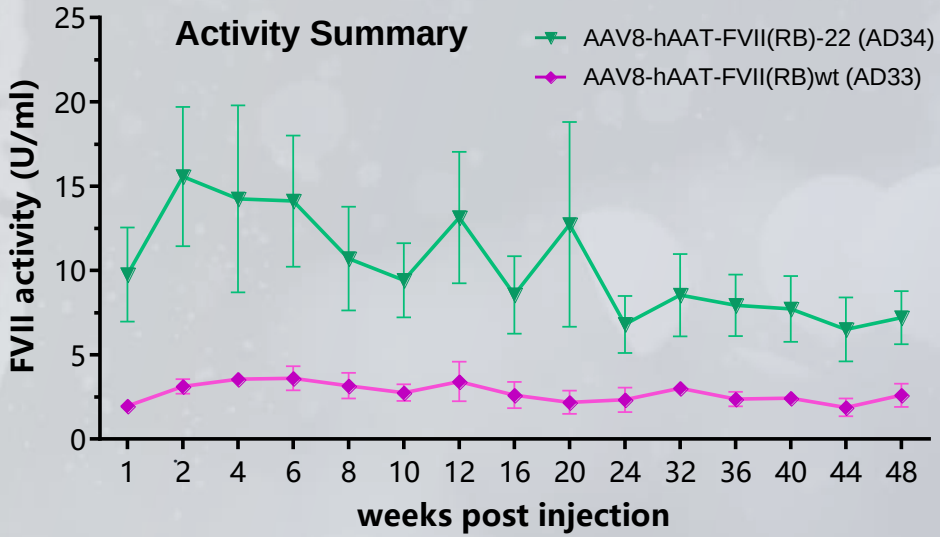
We leverage currently available capsids and promoters, which have proven successful in clinical trials, if they deliver sufficient expression of the desired protein to cure the disease of interest.

Our lead candidate demonstrated increased, stable expression levels and Factor VII activity for a sustained period of time without adverse effects.

SANAGEN

We develop novel capsids and promoters that are more specific for targeted cell types and/or have higher expression to achieve increased production of the therapeutic protein.





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Our novel promoter

Our novel capsid

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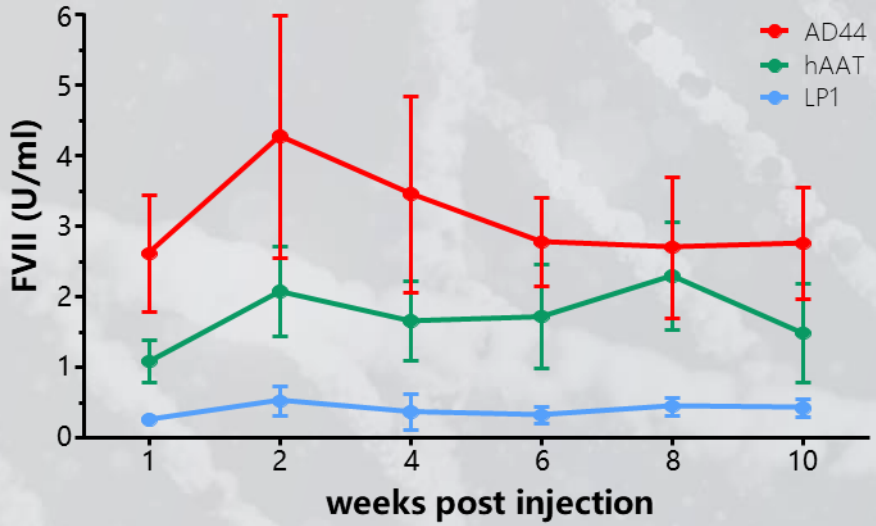

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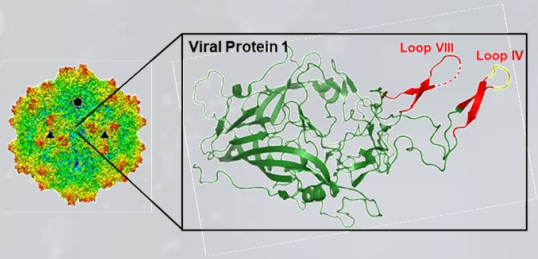
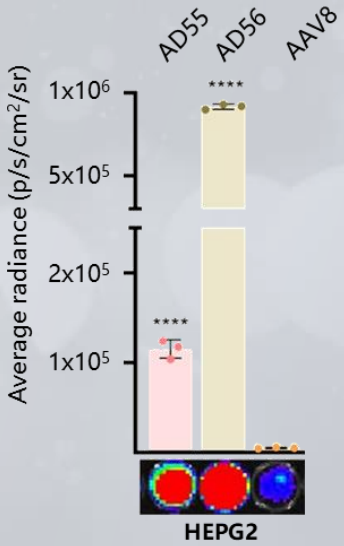
We develop novel capsids and promoters that are more specific for targeted cell types and/or have higher expression to achieve increased production of the therapeutic protein.

Our novel promoter showed a 2-fold improvement in expression and our novel capsid demonstrated up to 200-times higher expression in liver cells.





Selecting factor VII deficiency as initial target for our lead gene therapy candidate

Factor VII deficiency is a rare genetic bleeding disorder that puts patients at risk of experiencing prolonged, uncontrolled bleedings as early as in infancy. The severity varies greatly per patient but at least 20% of them experience life-threatening bleedings and related complications.

10K PATIENTS SUFFER FROM FACTOR VII DEFICIENCY

We selected factor VII deficiency as first target application for our gene therapy platform for technical and feasibility reasons:

Feasibility rationale

- High unmet need
- Severe economic burden
- Potential ODD benefits
- Our strong hemophilia expertise
- No direct competition

Technical rationale

- Well-defined disease
- Low expression required
- Current components sufficient
- Our variants improve treatment
- Proof of liver targeting

Factor VII deficiency represents an appealing beachhead indication

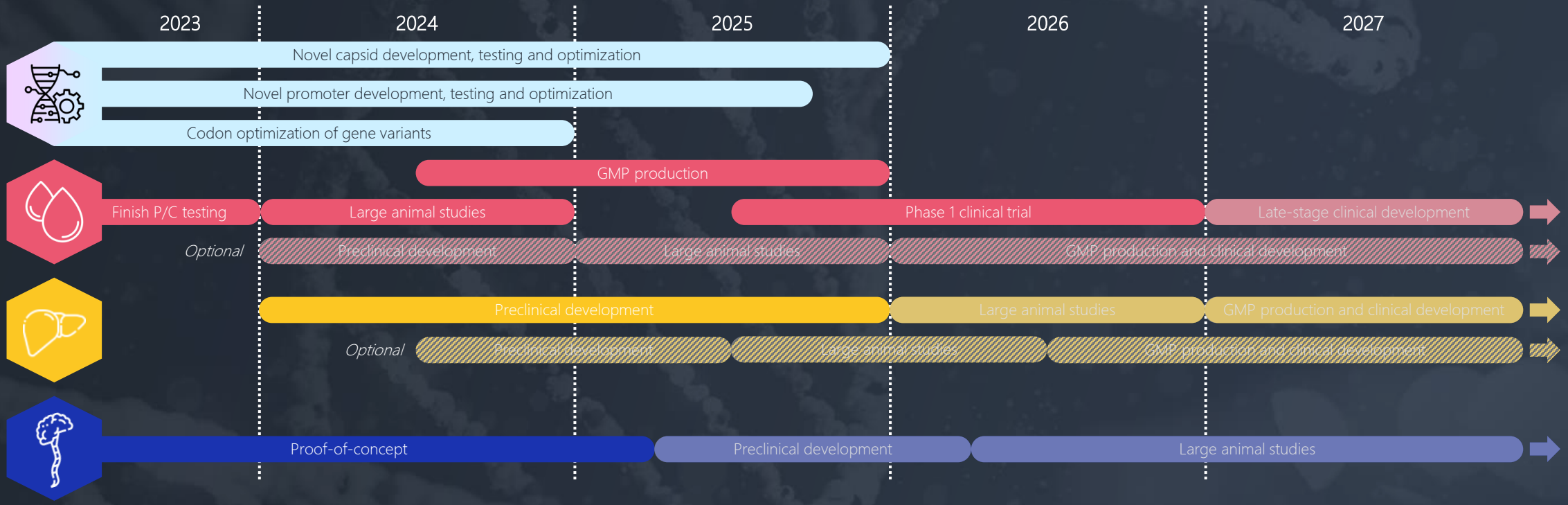
Factor VII deficiency is an ideal beachhead indication to de-risk the development of additional applications and open the broad market potential of our gene therapy platform.

BLEEDING DISORDERS
Factor X deficiency

OTHER LIVER-DIRECTED DISORDERS
Alpha-1 antitrypsin deficiency
Hereditary angioedema

NEURODEGENERATIVE DISEASES
Multiple Sclerosis

Convinced by our preclinical proof-of-concept for factor VII deficiency, we are ready to move our gene therapy towards the first patients via clinical trials



SANAGEN

Gene therapy platform
Factor VII deficiency
Factor X deficiency
Alpha-1 antitrypsin deficiency
Hereditary angioedema
Multiple sclerosis

An €8.0M investment will enable us to advance to the next value inflection points

Gene therapy platform

- Finish design of novel capsid and novel promoters
- Test novel capsids in AATD program
- Test novel promoters in all ongoing programs
- Optimize gene variant codons in all ongoing programs

Factor VII deficiency

- Finish preclinical development/proof-of-concept studies
- Conduct large animal studies
- GMP production of clinical gene therapy candidates
- Phase 1 clinical trial with factor VII deficiency patients

AATD & **MS**

- Establish proof-of-concept for AATD
- Establish proof-of-concept via research collaboration for MS

Additional funding will enable us to advance other gene therapy programs to establish a broad portfolio of gene therapy candidates, which will significantly increase our value.

SanaGen offers an attractive investment opportunity with significant exit potential

The value of gene therapies with successful early-stage clinical trials is huge. We foresee 3 exit opportunities for SanaGen:

ACQUISITION
of SanaGen or certain assets (gene therapy candidates)

OUT-LICENSING
of novel capsids and promoters

INITIAL PUBLIC OFFERING
of SanaGen to become a publicly-traded company

SanaGen at a glance: pioneering unique gene therapy approaches to provide a life-long cure for patients suffering from rare genetic diseases

Founded by experts in gene therapy and bleeding disorders, driven to make SanaGen and its gene therapies a success

We combine our unique gene therapy innovations with off-the-shelf components to develop our gene therapies

Building on promising PoC data, we move our lead gene therapy candidate for factor VII deficiency towards first patients

Targeting a rare bleeding disorder to de-risk our gene therapy platform and open up its use for other diseases

With gene therapies representing enormous value, we have an attractive exit opportunity for our company and its assets

For more information, please contact us:

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a life-long cure for patients suffering from genetic diseases