



NanoSyrinx

a novel platform technology
for **targeted intracellular**
drug delivery

ANGLO | LIFE SCIENCE
NORDIC | CONFERENCE

AngloNordic Life Science Conference
April 20th 2023, County Hall, London

NanoSyrinx today

Leadership Team



Joe Healey
Founder & CEO/CTO



James Lapworth
CBO/COO



Steve Taylor
Non-exec Chair



Marie McAvoy
CSO



Nick Waterfield
Co-Founder & SAB



Jane Dancer
NED



A seasoned leadership team including industry veterans from GSK, FujiFilm Diosynth, F-Star & MedImmune and experts in synthetic/molecular biology guiding our technical and strategic development.

- Discovery stage company established 2020
- Spinout from Waterfield Lab at Warwick Medical School
- Closed a £6.2M Seed+ round in July 2021

NanoSyrinx is thrilled to be backed by



The **un-druggable** cell

< 20% Human proteins 'drugged'
(mostly cell surface)



“Delivering [biologics] into cells is the **Holy Grail**...”

-- **morphosys**

“Delivering functional proteins to the interiors of cells would open up an entirely new range of targets for drug development...”

-- **MedImmune**

“Intracellular delivery is a strategic priority for us. We have already tried a number of solutions, including some quite ‘out there’ ideas, **but nothing really works.**”

-- **novo nordisk**

Analogous technologies demonstrate **market value** in **intracellular platform plays**



(Boston, MA)



(Oxford, UK)



(Boston, MA)



(Waltham, MA)

Technology

Protein nanoparticles

Exosomes

CPPs

Antibody-nucleotide conjugates

Application areas

Delivery of “compact” gene editing enzymes

Lysosomal storage disease, DMD and rare metabolic diseases

Enzyme replacement therapy, PPI inhibitors

RNA delivery for rare muscle disease (DM1)

A Round Stage Current Stage

Discovery stage

-Discovery stage →
-Preclinical (partnered)

-Discovery stage →
-IND enabling

-Discovery stage →
-Phase I for DMD

Funding

Feb 2023: \$193m (A&B)

Apr 2016: £10m
Sep 2018: £35.5m
Feb 2021: £69m

Oct 2016: \$0.6m
Dec 2018: \$59m
Mar 2021: \$116m
Oct 2021: \$181m (IPO)

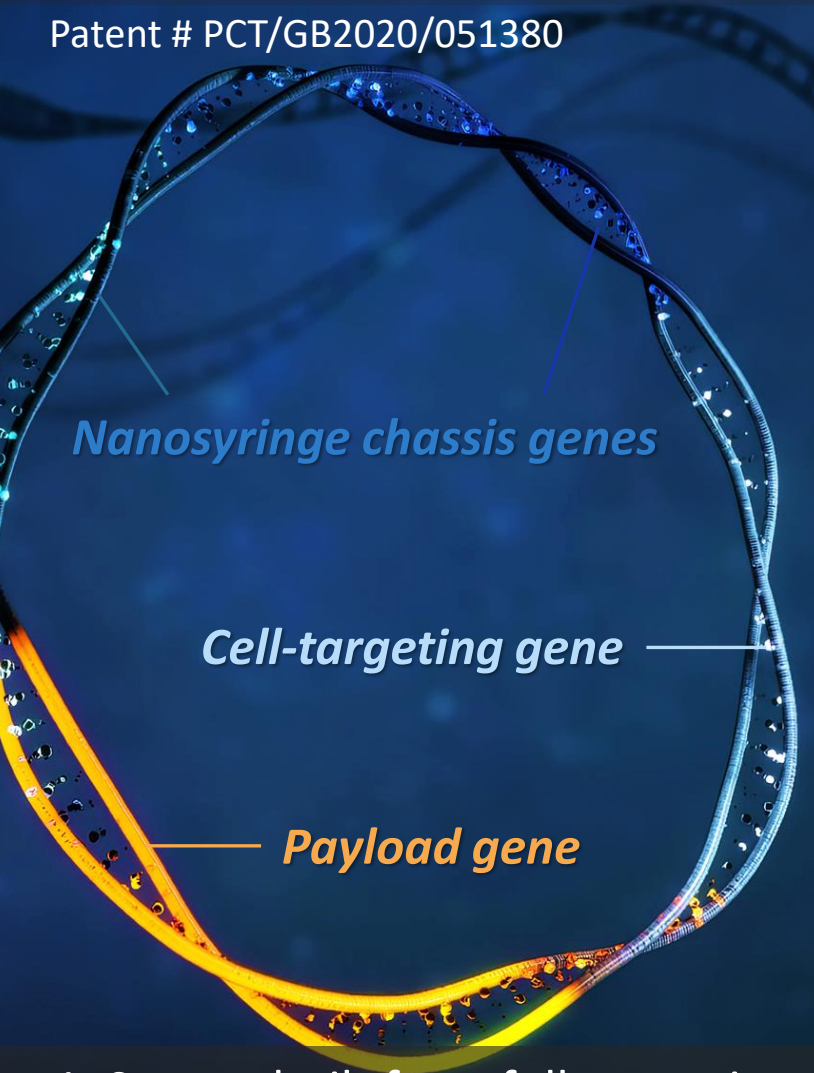
Apr 2019: \$50m
Aug 2020: \$115m
Sep 2020: \$268m (IPO)

Major investors

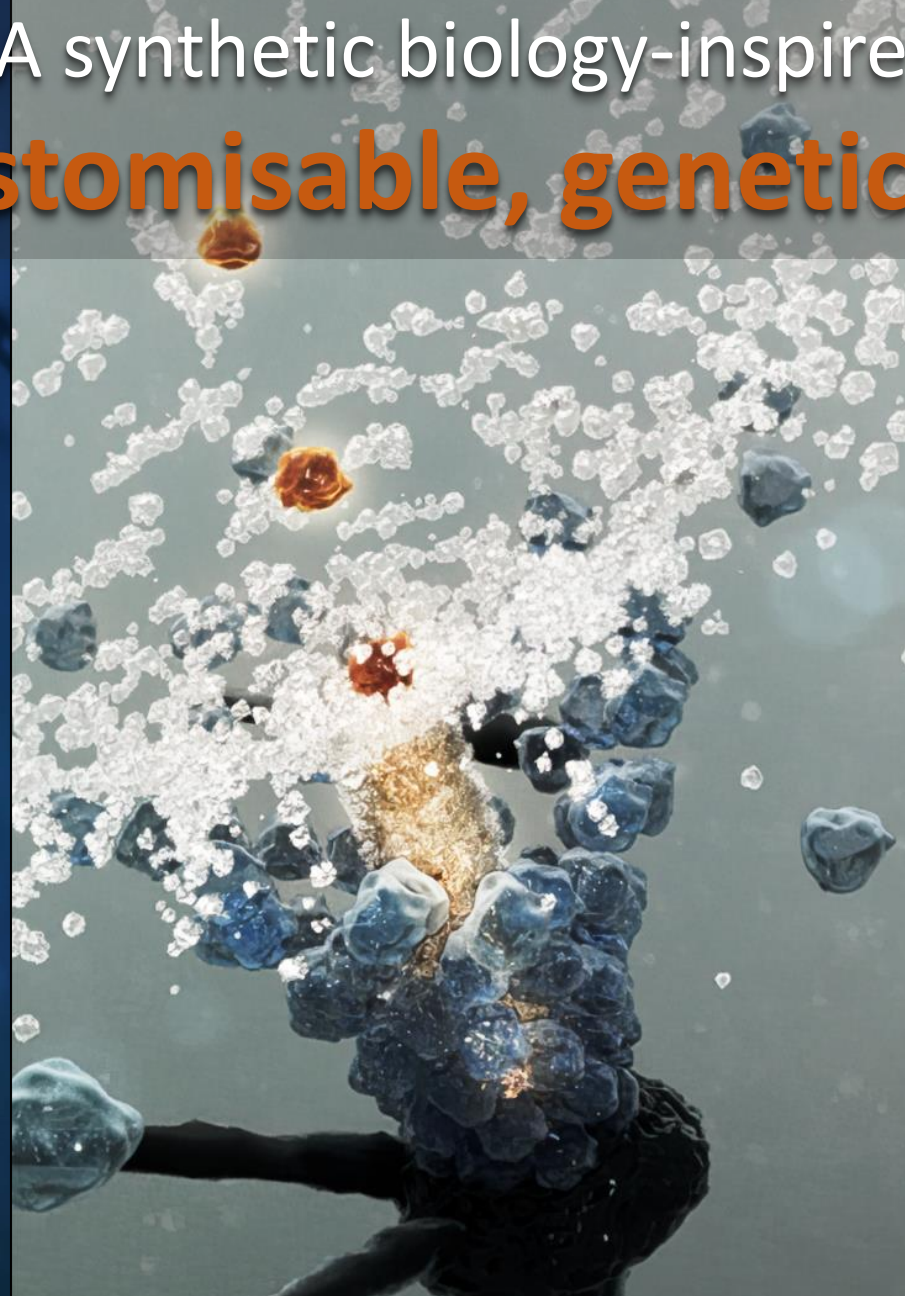


A synthetic biology-inspired,
fully customisable, genetic platform

Patent # PCT/GB2020/051380



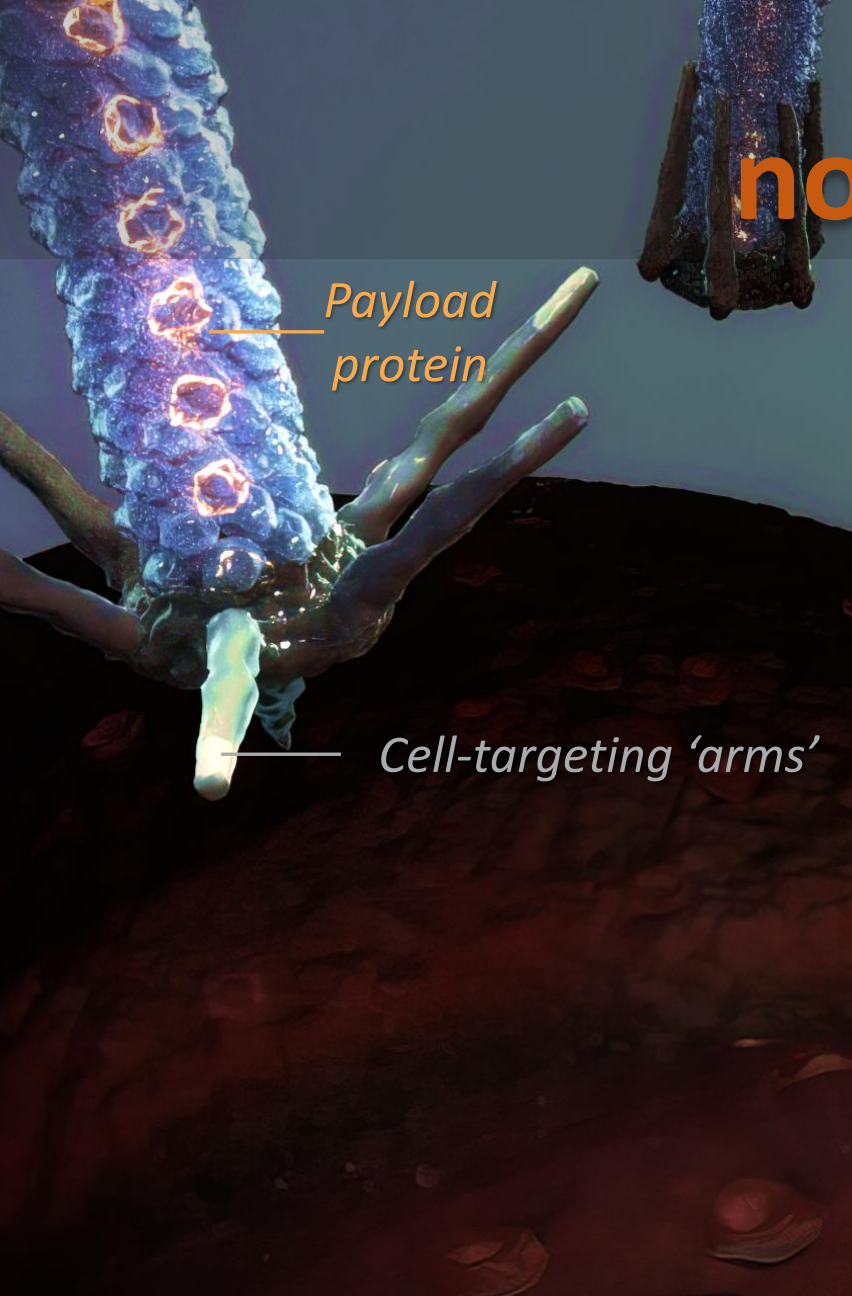
1. System built from fully genetic construct.



2. 'Single step' loading and assembly in *E. coli*



3. Nanosyringe complexes purified, loaded, ready for use

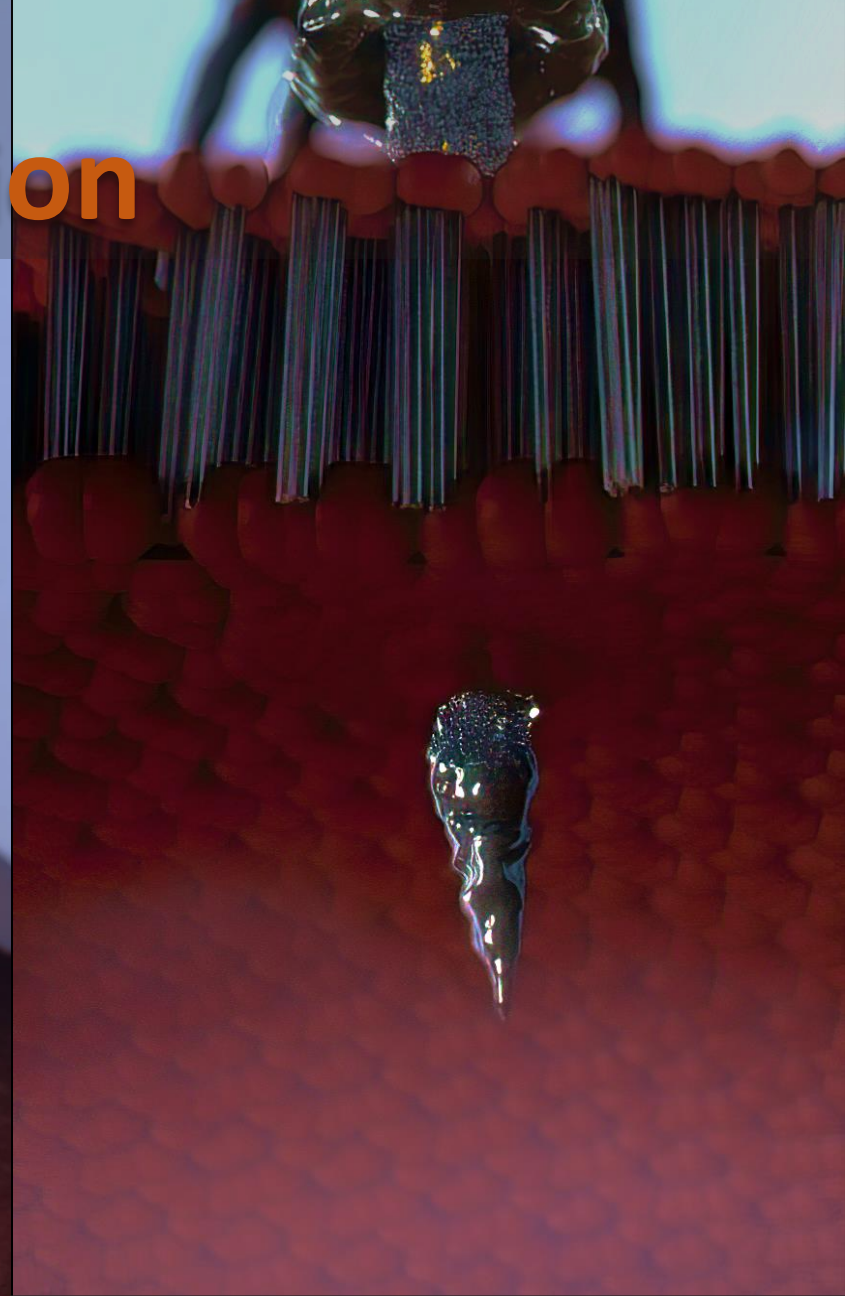


1. Loaded nanosyringes produced by out bacterial expression system

A completely
novel mode of action



2. Cell-targeting 'arms' selectively bind nanosyringe to cell surface



3. Nanosyringes actively pierce the membrane to deliver the 'API'

The competing technologies

No other technology in the marketplace offers this **unique combination of features.**

Value Point	Nanosyringes	Viral Vectors	Cell Penetrating Peptides	Exosomes/Liposomes	Antibody-Drug Conjugates
Inherently Targeted	✓	Partially		Partially	✓
Modifiable cargo	✓	✓	Varies	✓	✓
Stable	✓	Varies	Varies	Varies	Varies
Simple manufacture	✓		Often		
Platform Tech	✓	✓		✓	✓
'Active' delivery	✓				
Fully genetically controllable	✓	✓	Potentially		

NanoSyrinx **technology development**

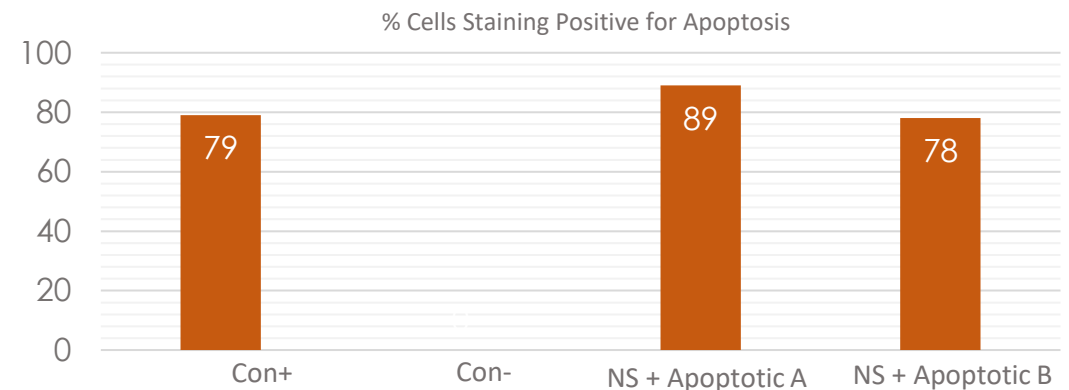
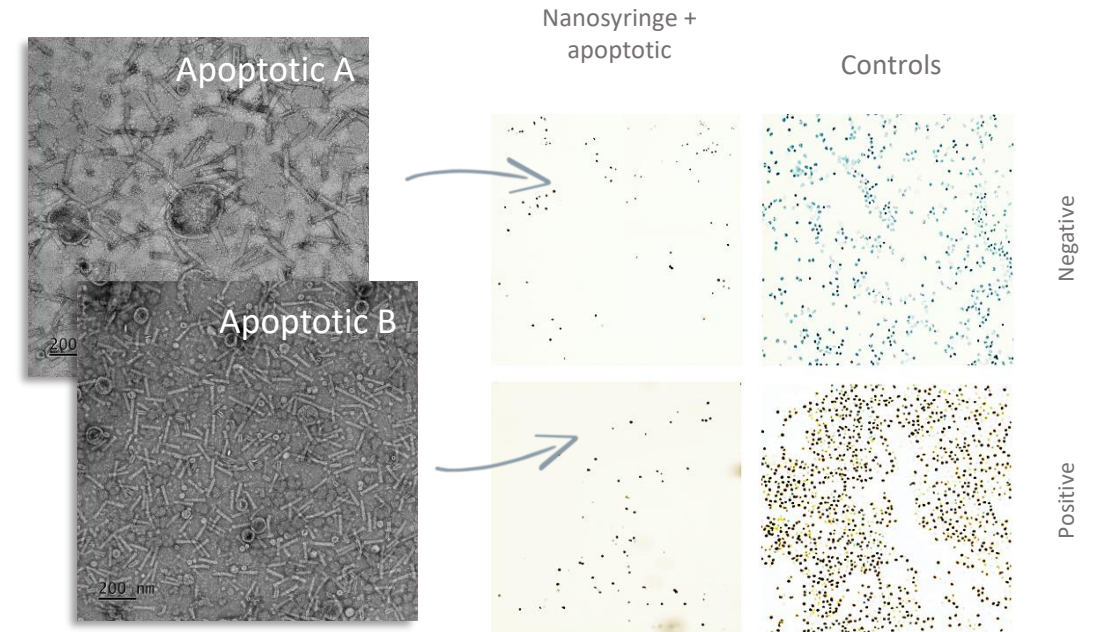
Key platform validation in hand or in progress:

- Control of payload loading ✓
- Delivery of diverse payloads ✓
- Feasible scalability ✓
- Ability to selectively target ✓

Patented core IP, with further IP under development.

Feasibility study in progress with a large pharma, yielding promising positive data.

Example Data: NanoSyringes are capable of delivering the small, human-derived pro-apoptotic peptides to **primary human cells**.



NanoSyrinx has a versatile platform with many application areas



In vivo Therapeutics

Utilising a suite of cytolethal/manipulatory payload options (e.g.):

- (Nano/Anti)bodies
- PROTACs
- Toxins/apoptotics

Reward

£££

Risk



Ex vivo Cell & Gene Therapies

Delivery of cell modulating and genetic engineering payloads (e.g.):

- Nucleases
- Cellular differentiation factors
- Antigens

££



In vitro Biotechnology Tools

Utilising a suite of useful probes and engineering tools (e.g.):

- Reporter proteins
- Probes
- Enzyme delivery (e.g. Cas9)

£

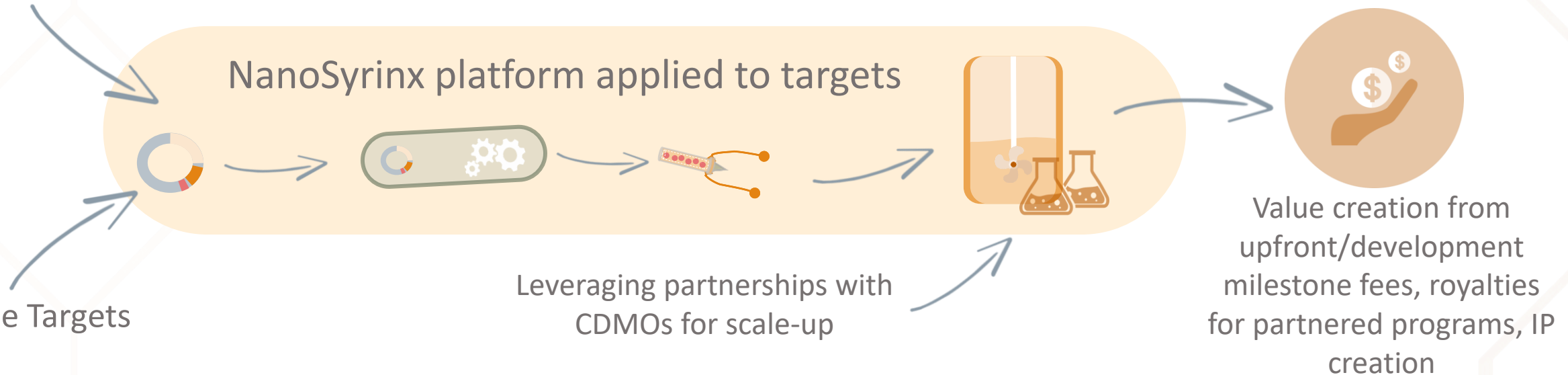


Multiple avenues for **value creation**

NanoSyrinx proposes a **hybrid model**:

- develop **in-house programs** for currently undruggable targets and tools
- collaborative **development and discovery** on **partner** targets

Partner Targets



Leveraging partnerships with
CDMOs for scale-up

Value creation from
upfront/development
milestone fees, royalties
for partnered programs, IP
creation

Co-development partnership deal precedents in the space

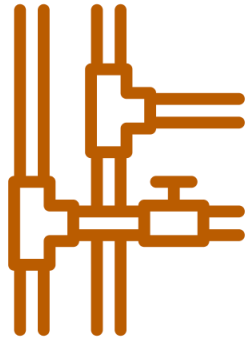


Announced	Jan 2023	Feb 2023	Mar 2020	June 2020
Scope	Development of AAV capsids for IV delivery of gene therapies to the CNS Preclinical and later development to be led by Lilly (& Prevail subsidiary)	Development of AAV capsids for ocular delivery of up to 3 gene therapies AbbVie will lead on payloads, clinical development & commercialisation	Development of delivery candidates for up to 5 rare disease targets All clinical development to be done by Takeda	Development of delivery candidates for up to 5 CNS targets All in vivo and clinical development to be done by Lilly
Terms	\$55m upfront + equity \$685m in R&D & commercial milestones	\$70m upfront \$595m in option fees and R&D milestones Undisclosed commercial milestones	\$44m upfront and near-term milestones \$840m in development milestones	\$20m upfront \$10m investment \$1.2Bn development milestones
Source	https://www.fiercebiotech.com/biotech/lilly-seeking-better-cns-gene-therapies-pays-55m-join-abbvie-aav-specialists-list-partners	https://www.fiercebiotech.com/biotech/capsida-reels-another-big-pharma-deal-time-diving-eye-disease-abbvie	https://www.evoxtherapeutics.com/News/March-2020/Evox-Therapeutics-and-Takeda-collaboration	https://www.evoxtherapeutics.com/News/June-2020/Evox-Therapeutics-Enters-Into-Lilly-Collaboration

NanoSyrinx **in 2023**



Raising **£30M Series A**
(targeting close Nov '23)



3 Year program delivering **a**
therapeutic pipeline



Positioning to unlock multiple **high**
value co-development partnerships

NanoSyrinx

www.nanosyrinx.com

Want to learn more?



joe@nanosyrinx.com

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