NanoSyrinx

a novel platform technology for targeted intracellular drug delivery

www.nanosyrinx.com

NanoSyrinx today

Leadership Team

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Tony Johnson, MD
NED
Goldfinch Bio the kidney company AstraZeneca OrbiMed Healthcare Fund Management

- Discovery stage spin out from the Waterfield Lab at Warwick Medical School
- >£17M raised to date (most recently £10M in Aug 2024)

Our backers:

BGF
M. VENTURES
octopus ventures
Lilly
meltwind
UK INNOVATION & SCIENCE SEED FUND
Pioneer
IQ CAPITAL

The "undruggable cell" problem

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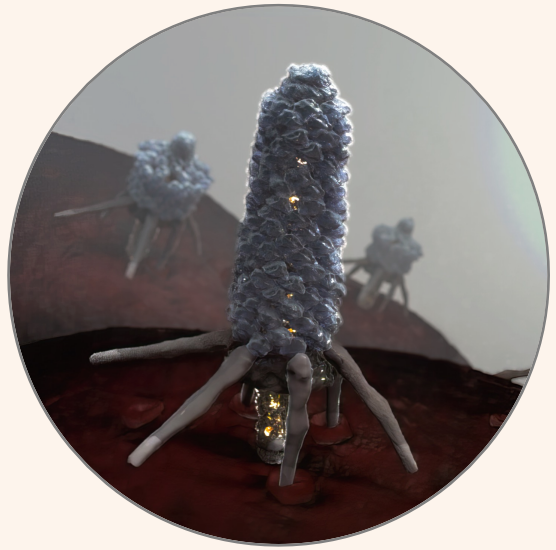
85%

of proteins considered "undruggable" using existing therapeutic approaches.

Small molecules are unable to address many classes of target.

Larger, more complex molecules fail to traverse the cell membrane.

Lack of selectivity limits therapeutic index & risks toxicity.



Targeted intracellular drug delivery is an unsolved problem, and our nanosyringe technology is the solution.

Our Vision

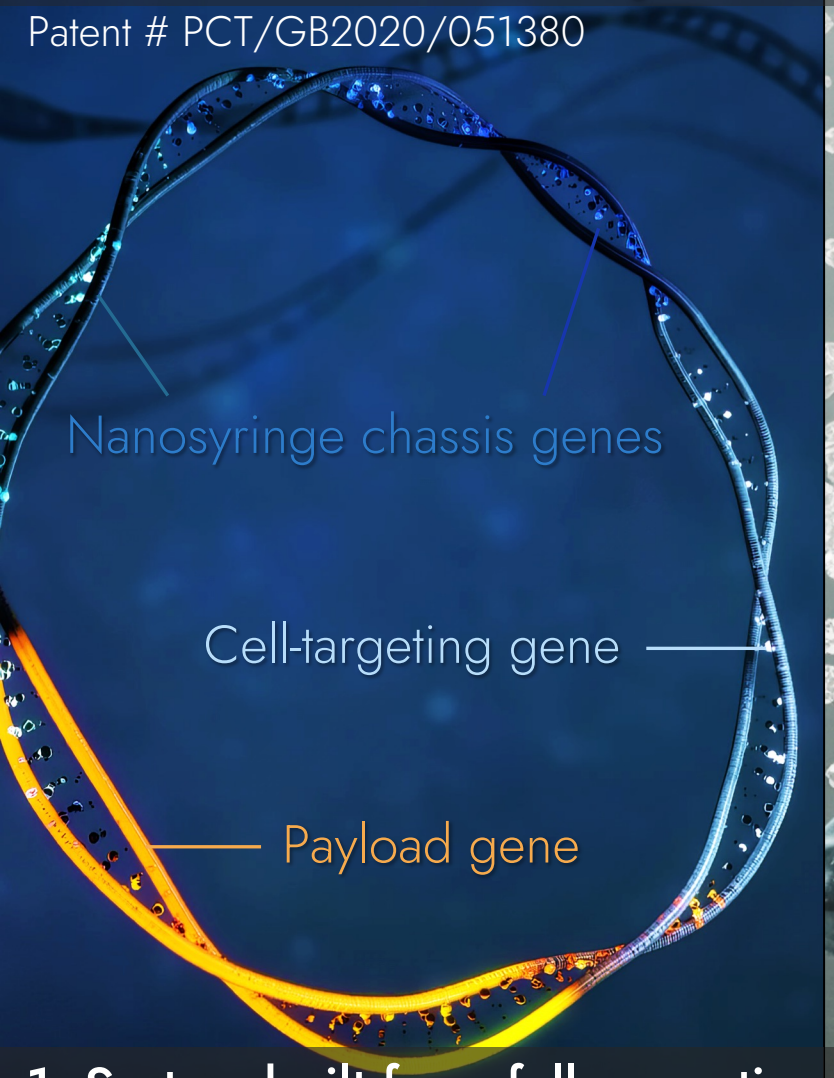
Our vision at NanoSyrinx is to unlock the interior of the cell and the myriad therapeutic opportunities within that are currently difficult (or impossible) to drug, by enabling targeted, intracellular delivery of protein therapeutics.

'Delivering the future of intracellular medicine'

A fully customisable, genetic platform

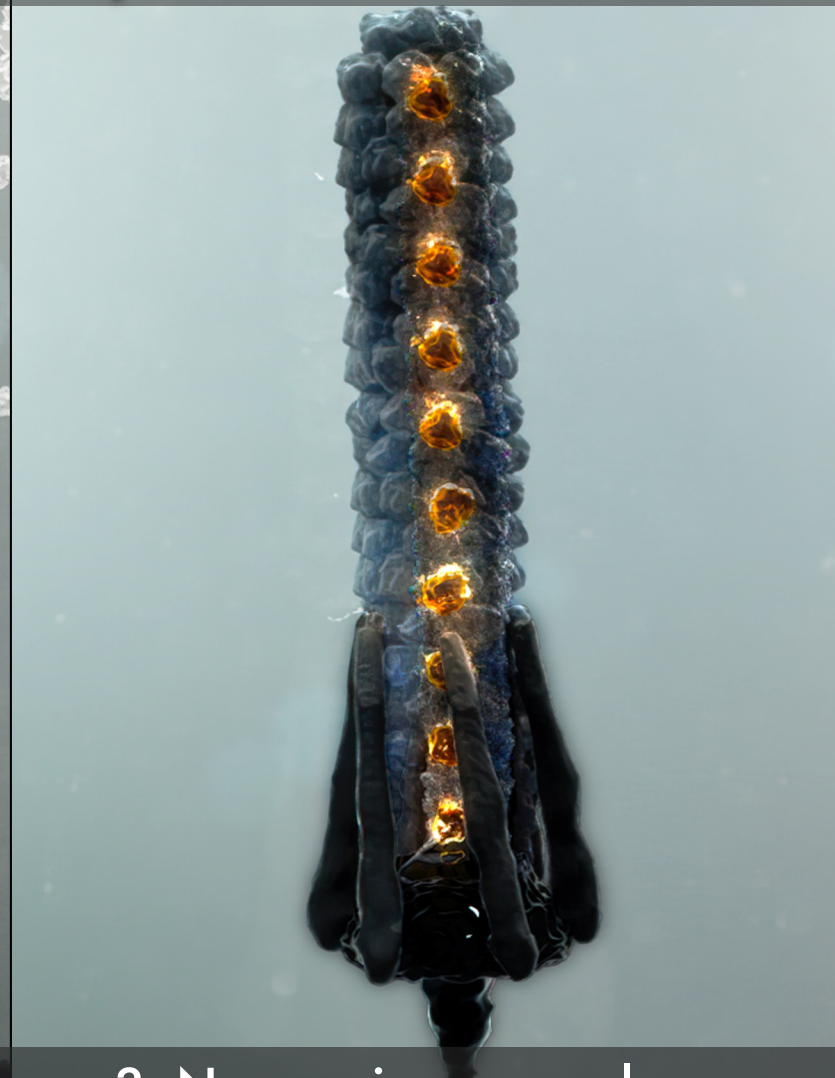
inspired by nature, perfected by us...

Patent # PCT/GB2020/051380



1. System built from fully genetic construct.

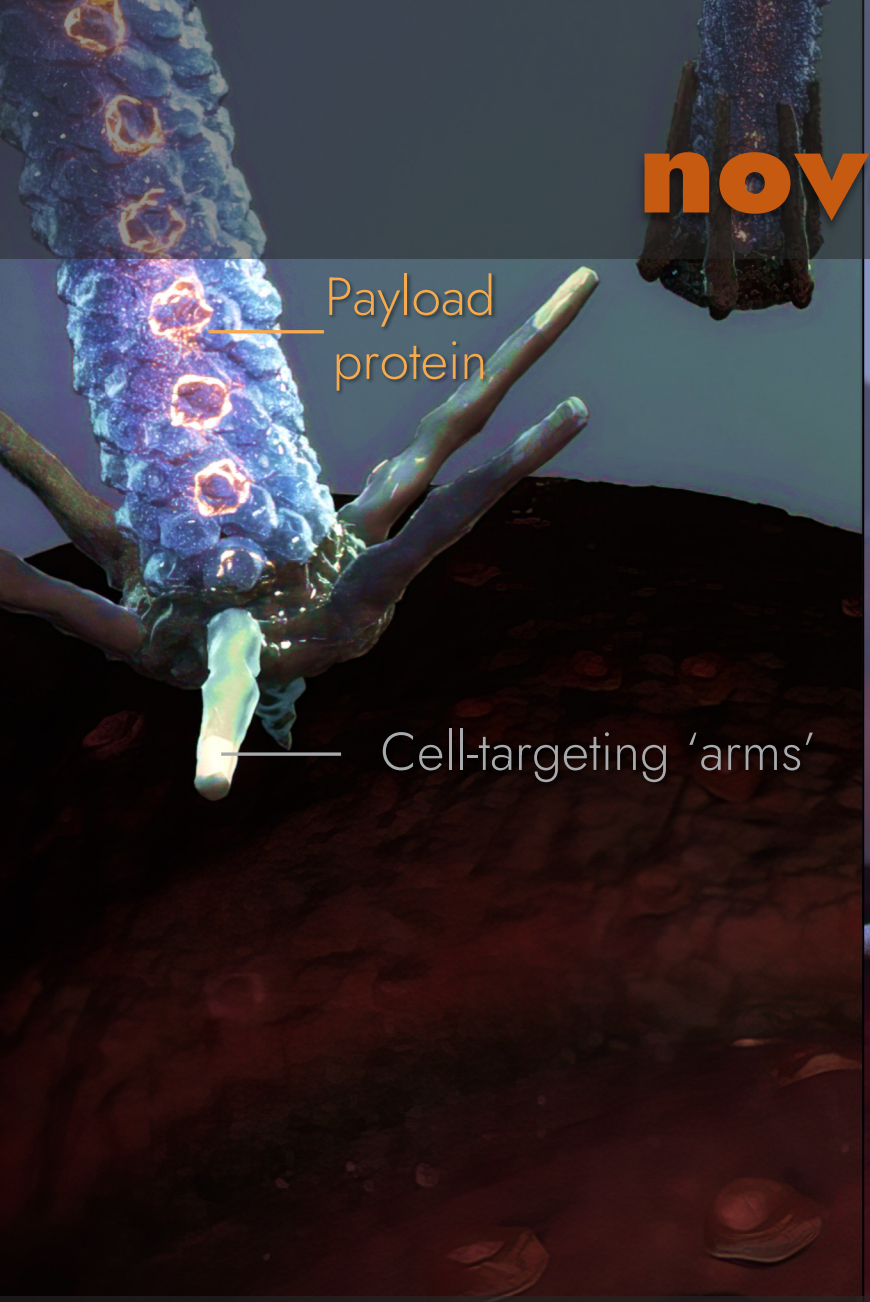
2. ‘Single step’ loading and assembly in E. coli

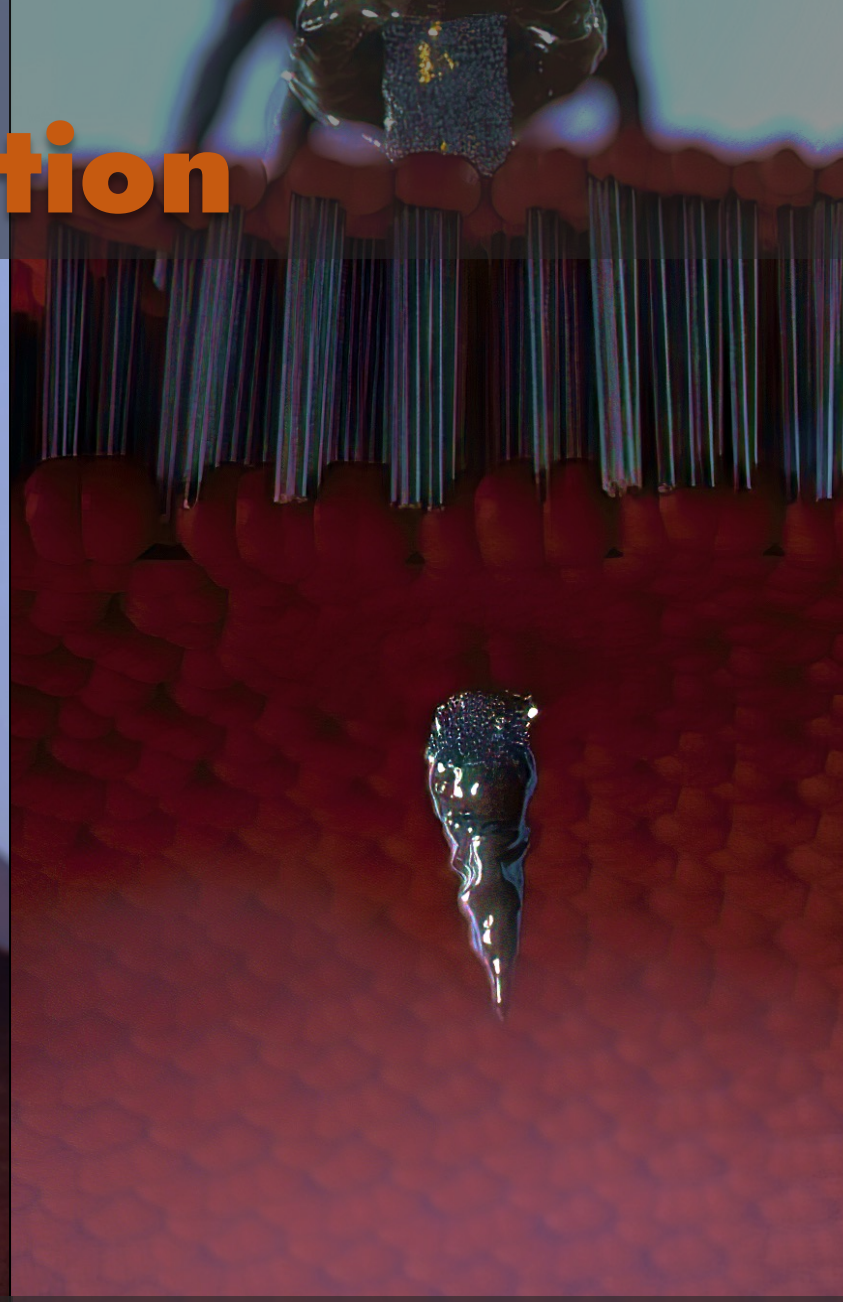


3. Nanosyringe complexes purified, loaded, ready for use

...with a completely

novel mode of action





1. Loaded nanosyringes produced by out bacterial expression system

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

3. Nanosyringes actively pierce the membrane deliver the ‘API’

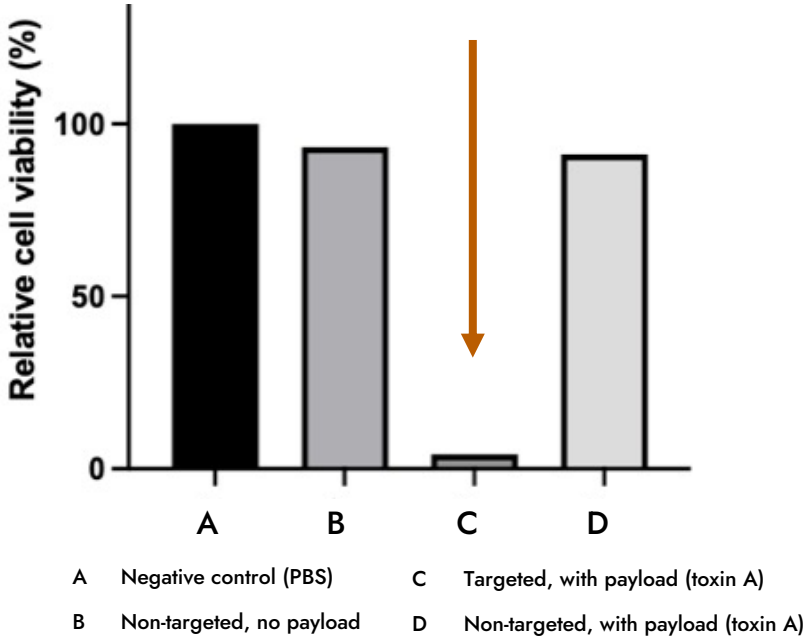
NanoSyrinx technology development

Internal and external validation demonstrates the potential of nanosyringes as targeted delivery vehicles.

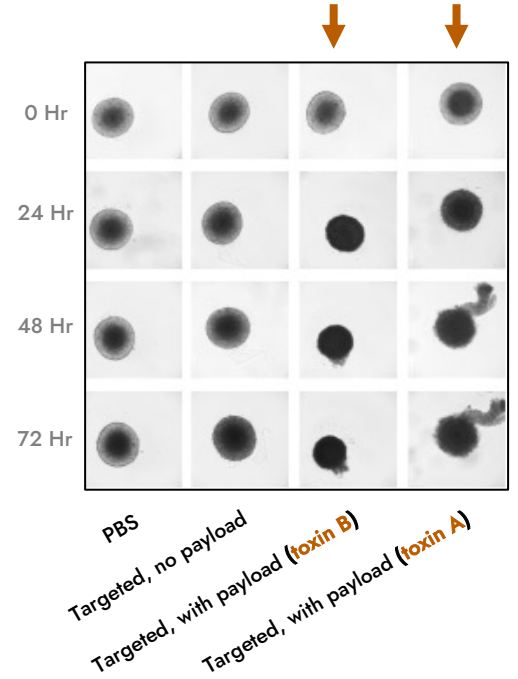
Key platform validation in hand or in progress:

- Control of payload loading ✓
- Ability to selectively target ✓
- Delivery of diverse payloads ✓
- *In vitro* validation ✓
- *In vivo* validation underway

Potent cell-killing observed only when targeted and bearing cytotoxic payload



Nanosyringes also act selectively and effectively in 3D spheroid models

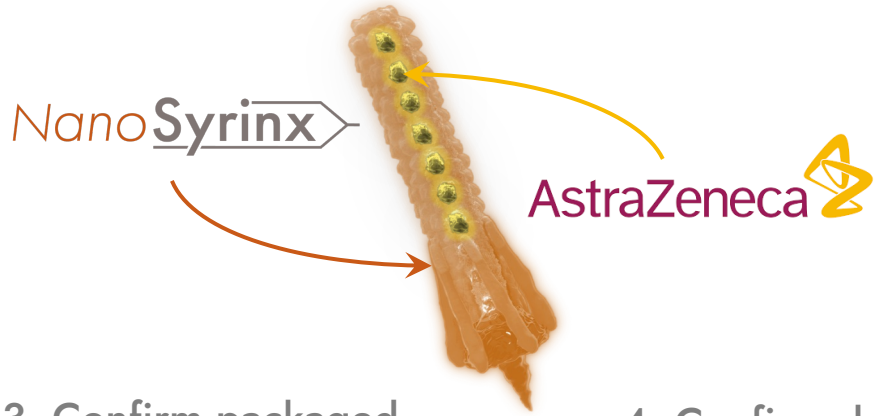


Example Data:
Nanosyringes can be retargeted and reloaded with new molecules to effect delivery against specific cell types with substantial total cell killing and nanomolar potency (depending on payload).

Nanosyringes also retain their targeted delivery properties in 3D culture, effectively killing/shrinking spheroids, reaching 80% of their maximal effect in 24 hours.

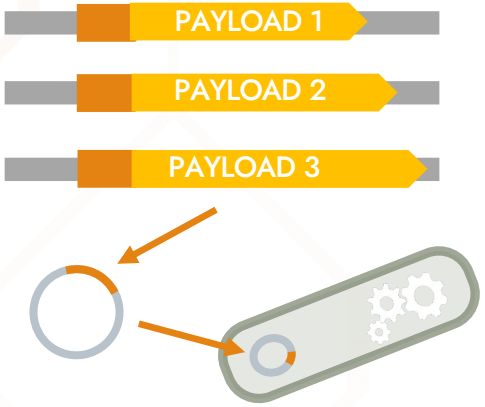
Delivering **partner payloads** against "undruggable" targets

We have successfully delivered a proof-of-concept collaboration with AstraZeneca demonstrating that we can incorporate and deliver their payloads.

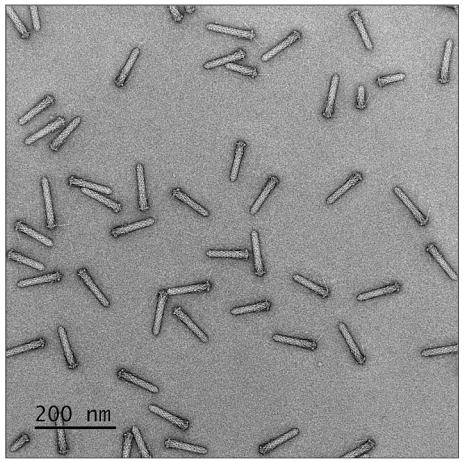


Example workflow:

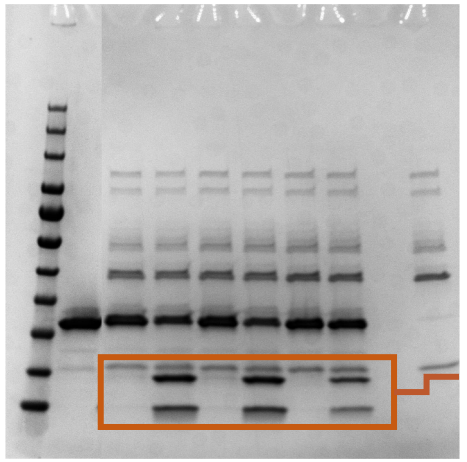
1. Clone partner payload into proprietary genetic platform



2. Confirm expression/ loading/assembly

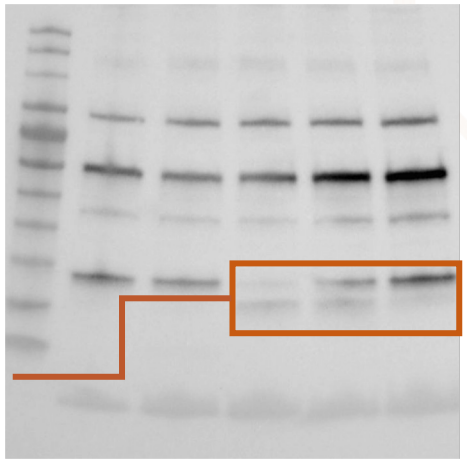


3. Confirm packaged payload is functional



Target protein cleaved

4. Confirm delivery of active payload in cells



Delivery of a functional enzyme degrader of an "undruggable" intracellular oncology target produces a measurable knockdown in protein abundance (and downstream signalling).

Multiple options for value creation

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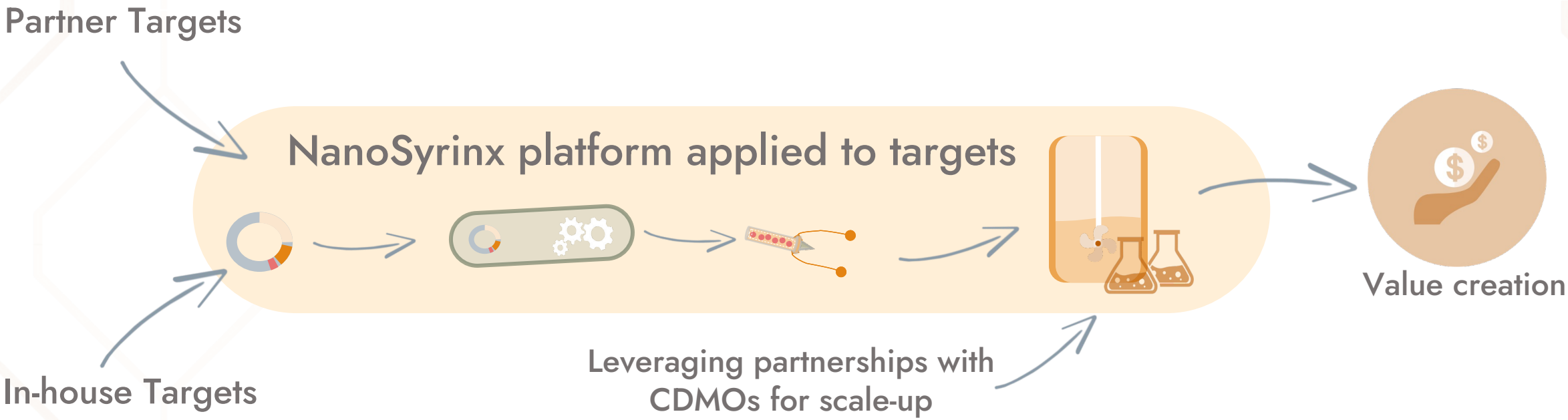
NanoSyrinx is pursuing a hybrid model:

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



Co-development deal precedents in the space

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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/Jun-2020/Evox-Therapeutics-Enters-Into-Lilly-Collaboration

NanoSyrinx **outlook**

NanoSyrinx

NanoSyrinx is on a mission to revolutionise biologics delivery, to enable a new generation of precision biotherapeutics.

We will do this over the next 2 years by delivering:

- **PoC in differentiated platform applications**
- **Platform biodistribution and dosing data**
- **Demonstrated scalability with CDMOs**

Positioning to capitalise on **traction with pharma** and **drive ambitious growth plans**

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Want to learn more?

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