



# Delivering the Future of Intracellular Medicine

[nanosyrinx.com](http://nanosyrinx.com)





Company Leadership



**Tom Farrell**  
CEO



**Joe Healey**  
Founder



**James Lapworth**  
CBO



**Marie McAvoy**  
CSO



**Chris Poole**  
CFO



Discovery stage spin out from the Waterfield Lab at **Warwick Medical School**

>£18M in equity financing raised to date

Based in Warwickshire, UK, with a headcount of 19

Independent Board Members



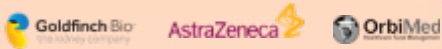
**Edwin Moses**  
Non-exec Chair



**Jane Dancer**  
NED



**Tony Johnson, MD**  
NED





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*



# The “undruggable cell” problem

85%

of proteins considered  
“undruggable” using  
existing therapeutic  
approaches.



Small molecules are unable to  
address many classes of target



Large molecules fail to traverse  
the cell membrane



Lack of selectivity limits  
therapeutic index & risks toxicity

## ***The solution***

Targeted intracellular  
drug delivery is an  
**unsolved problem**,  
and our Nanosyringe  
platform offers the  
solution.





# How Nature Solved **Biologic Delivery**



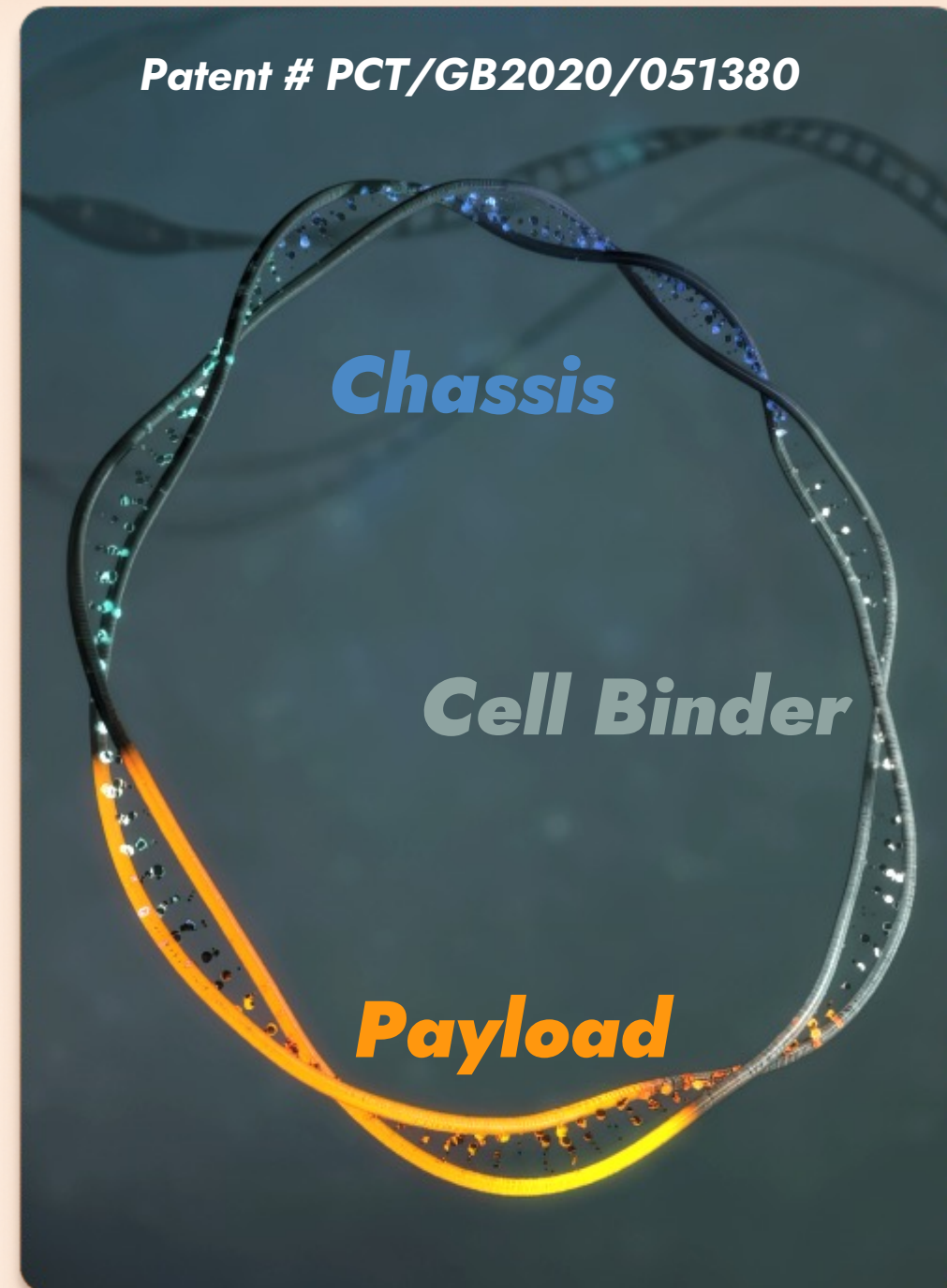
## ***Inspired by Nature, Engineered for Medicine***

Nanosyringes are **naturally-occurring** protein delivery systems evolved in bacteria to target specific cells making them **ideal for delivering complex therapeutics**.

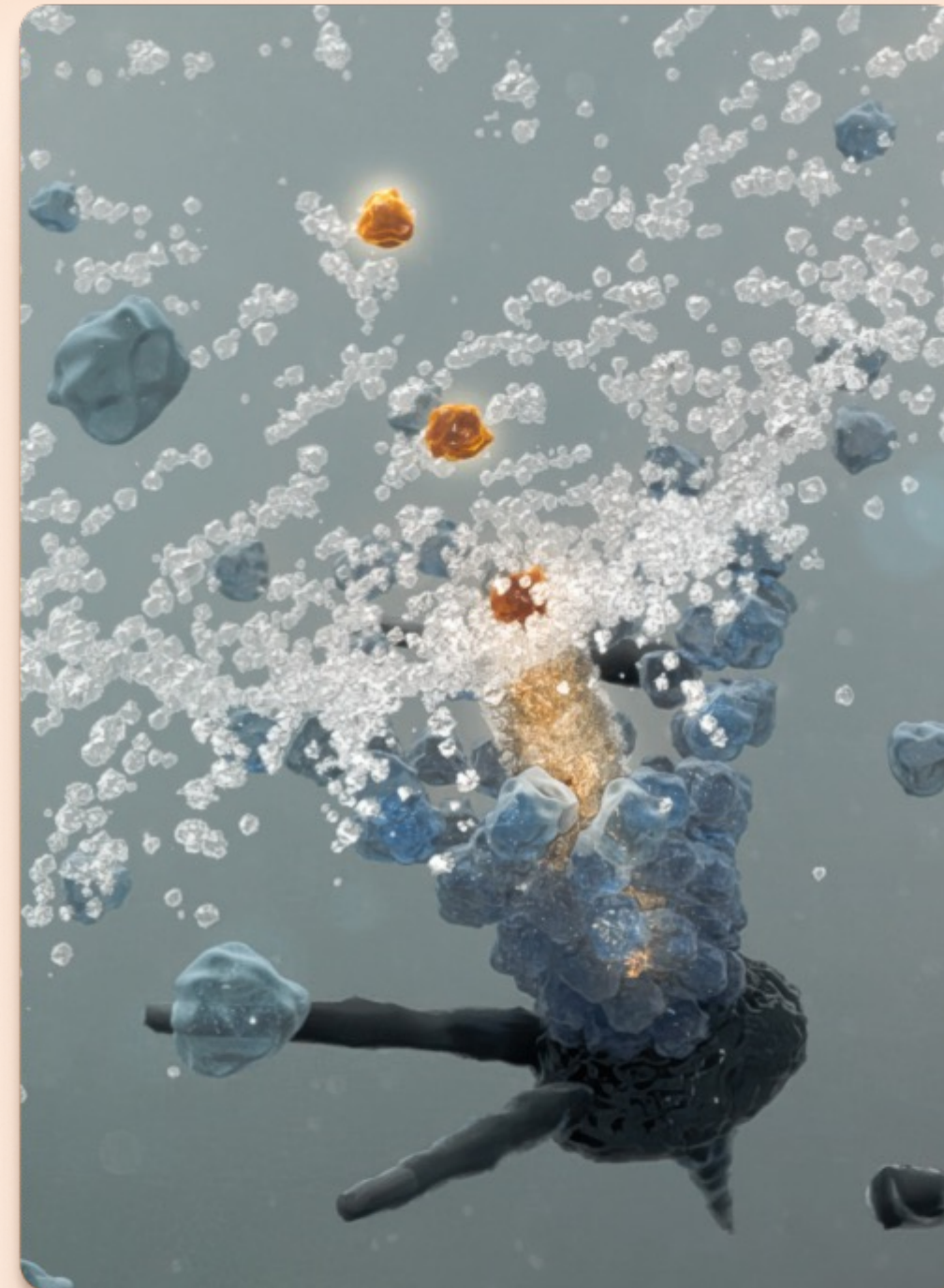
Through synthetic biology, we can harness and refine these systems to deliver complex therapeutic molecules **directly to the cytosol in a targeted manner**—something traditional drug delivery methods struggle to achieve.



# A **synthetic biology-inspired**, fully customisable, genetic platform



**1** System built from a fully genetic construct.



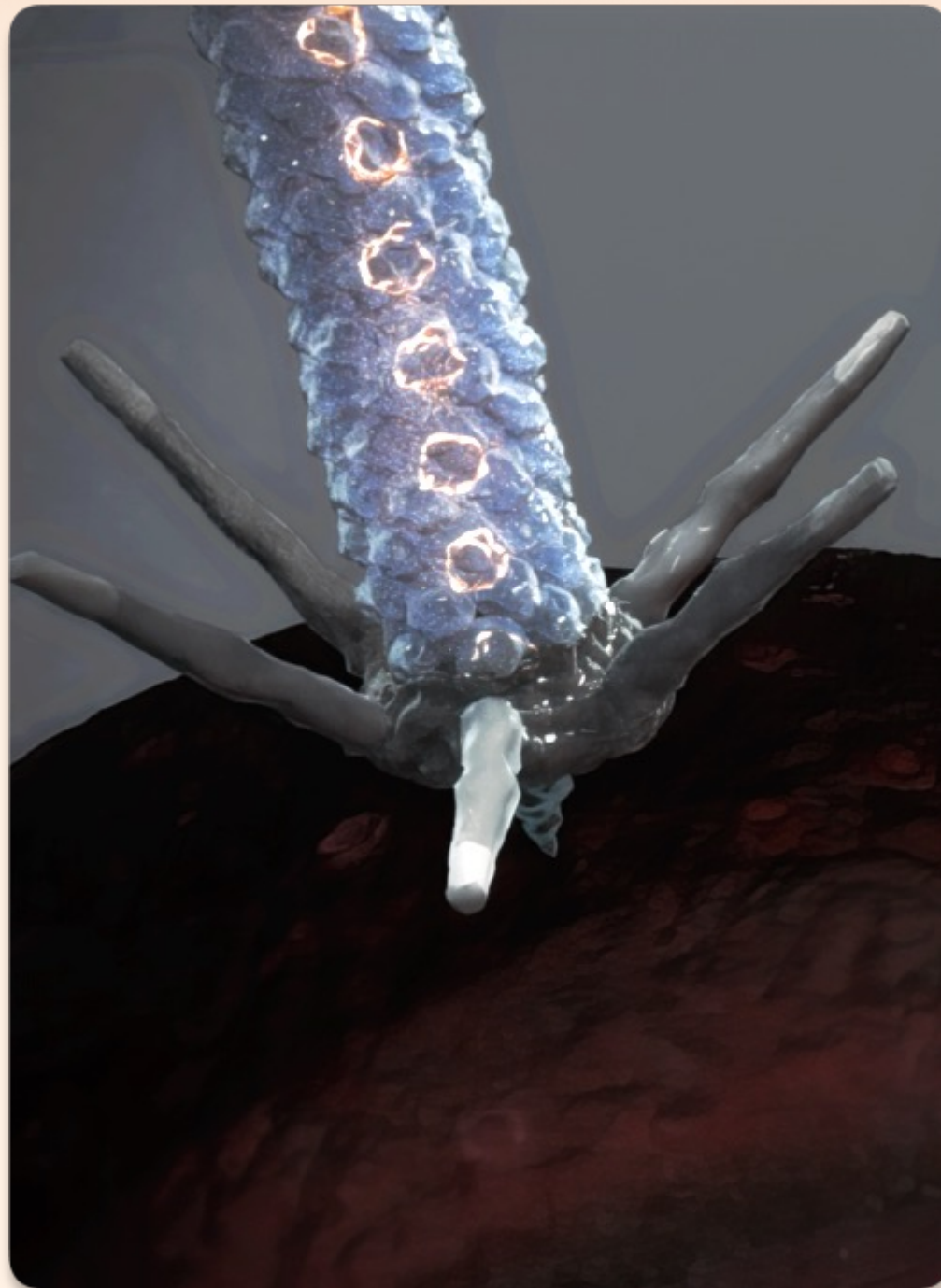
**2** 'Single step' loading and assembly in E. coli



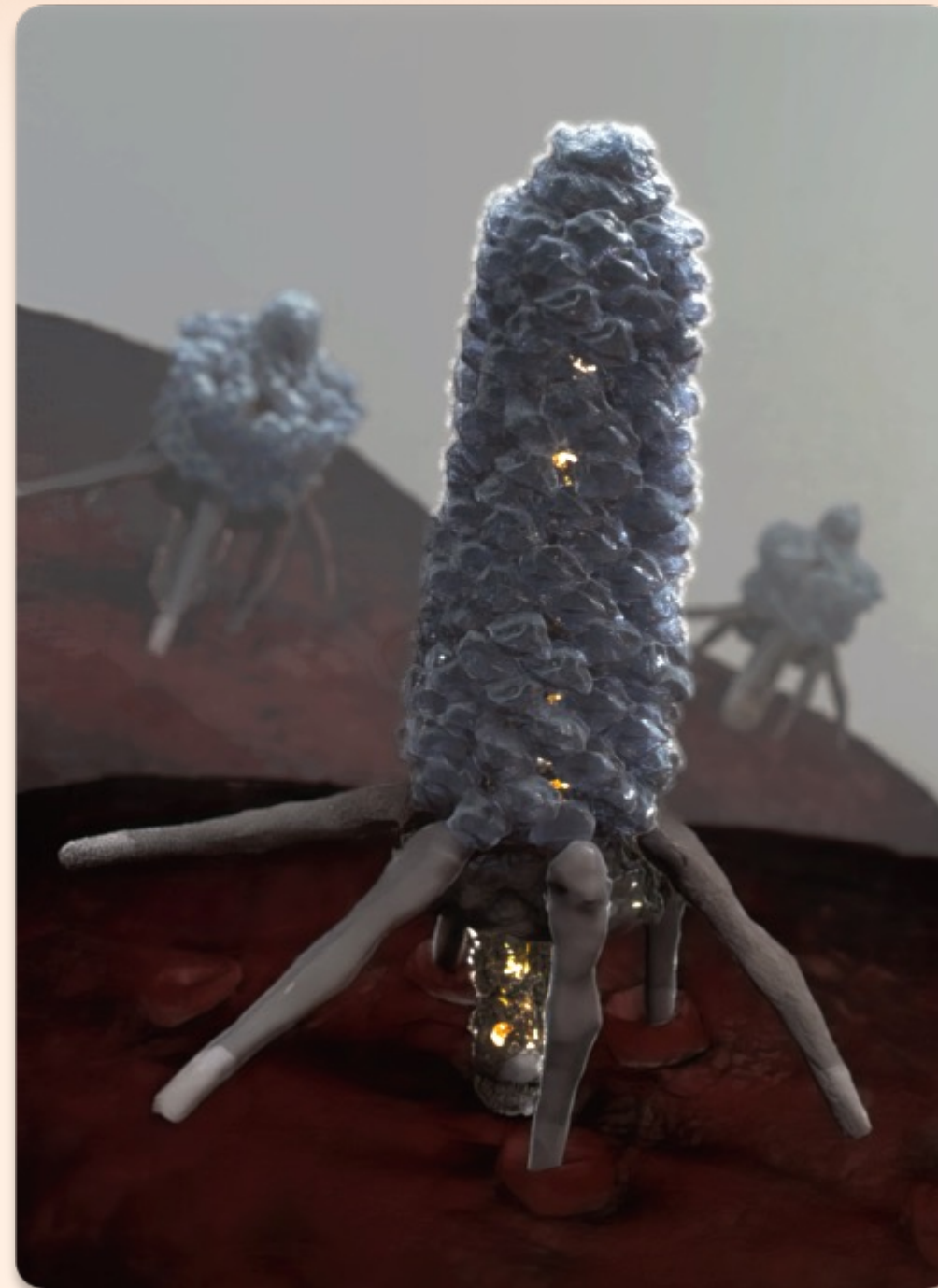
**3** Nanosyringe complexes purified, loaded, ready for use



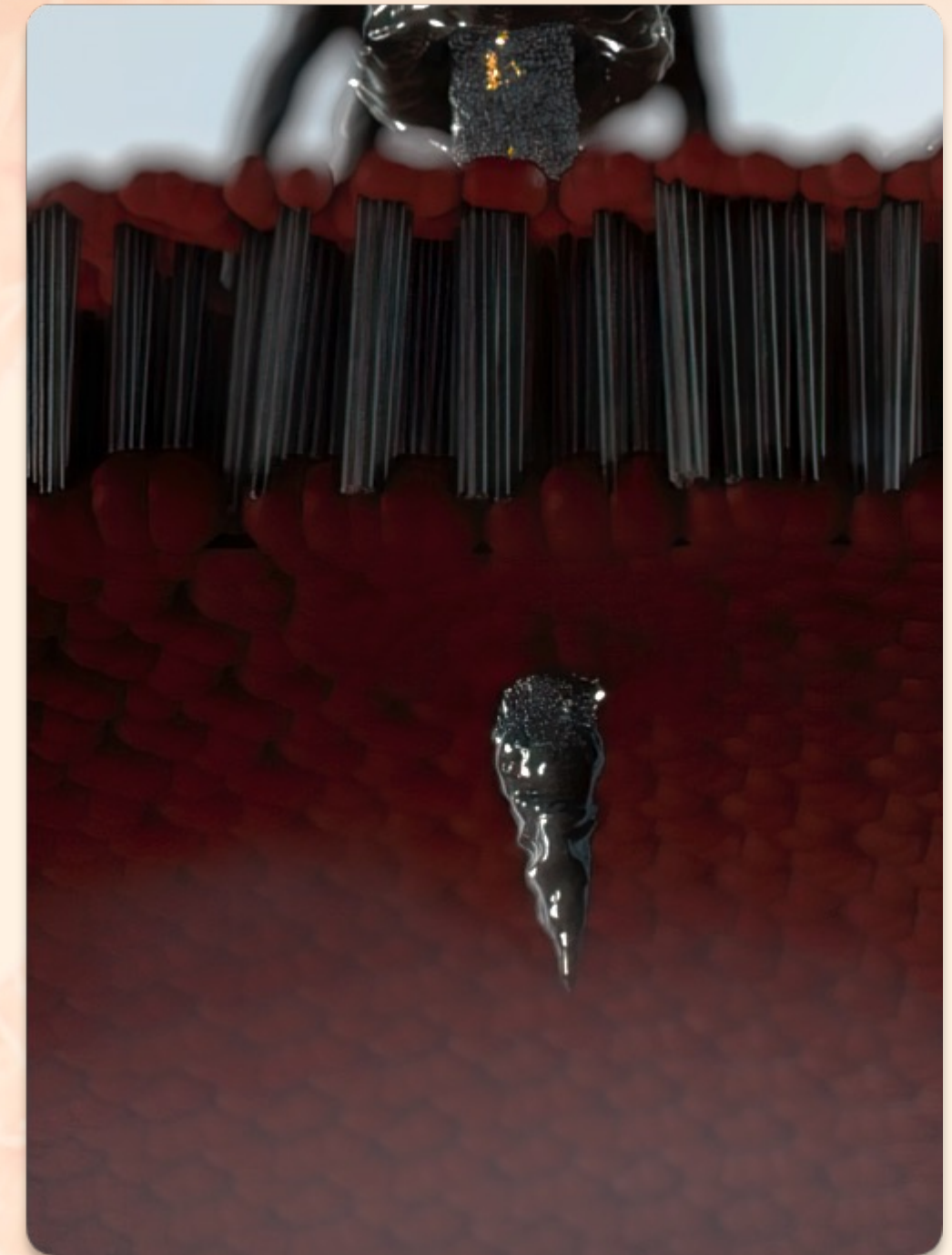
...with a completely novel **mode of action**



**4** Purified Nanosyringes administered in relevant setting



**5** Cell-targeting 'arms' selectively bind Nanosyringe to cell surface

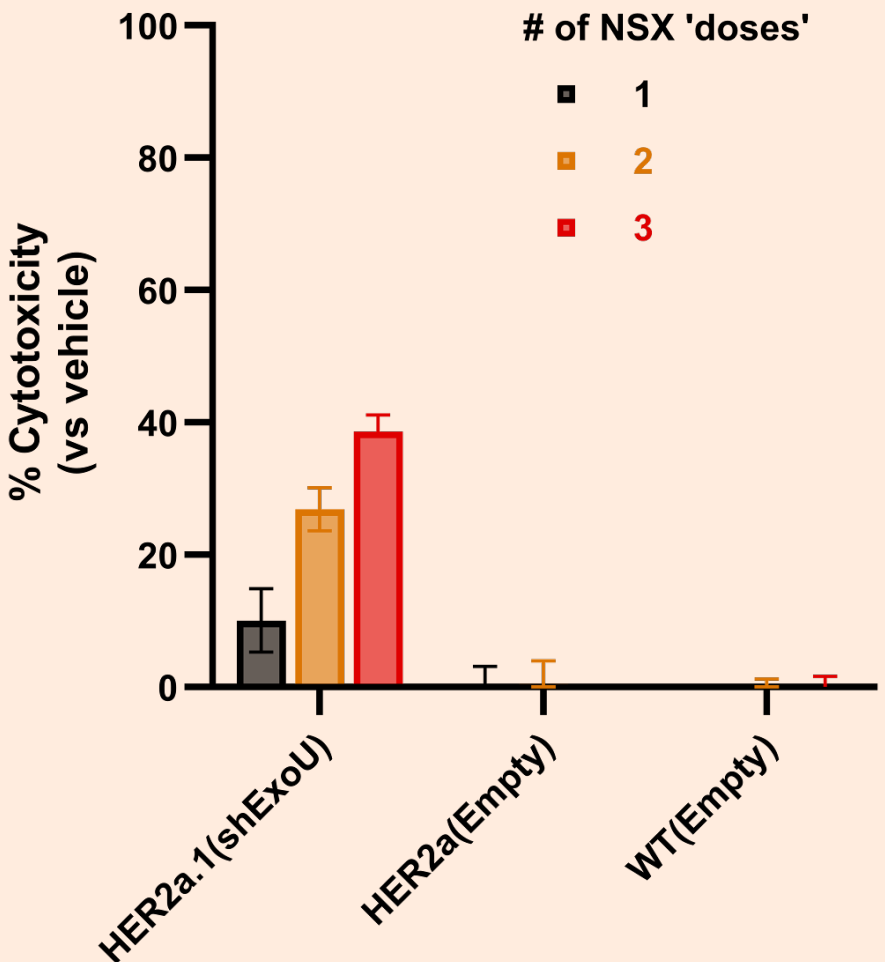
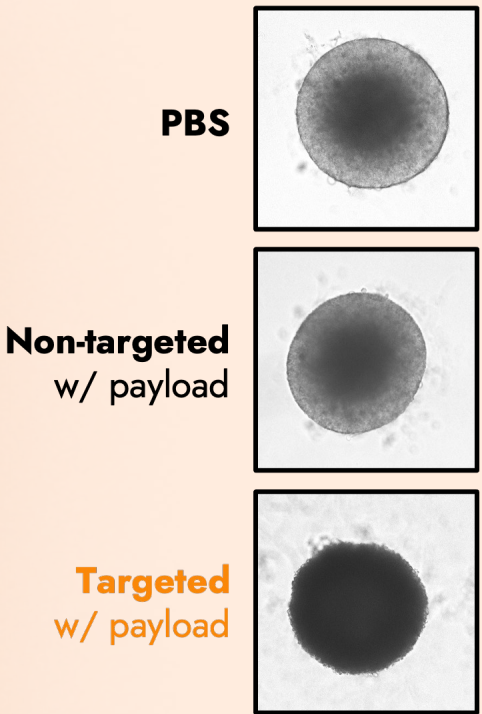
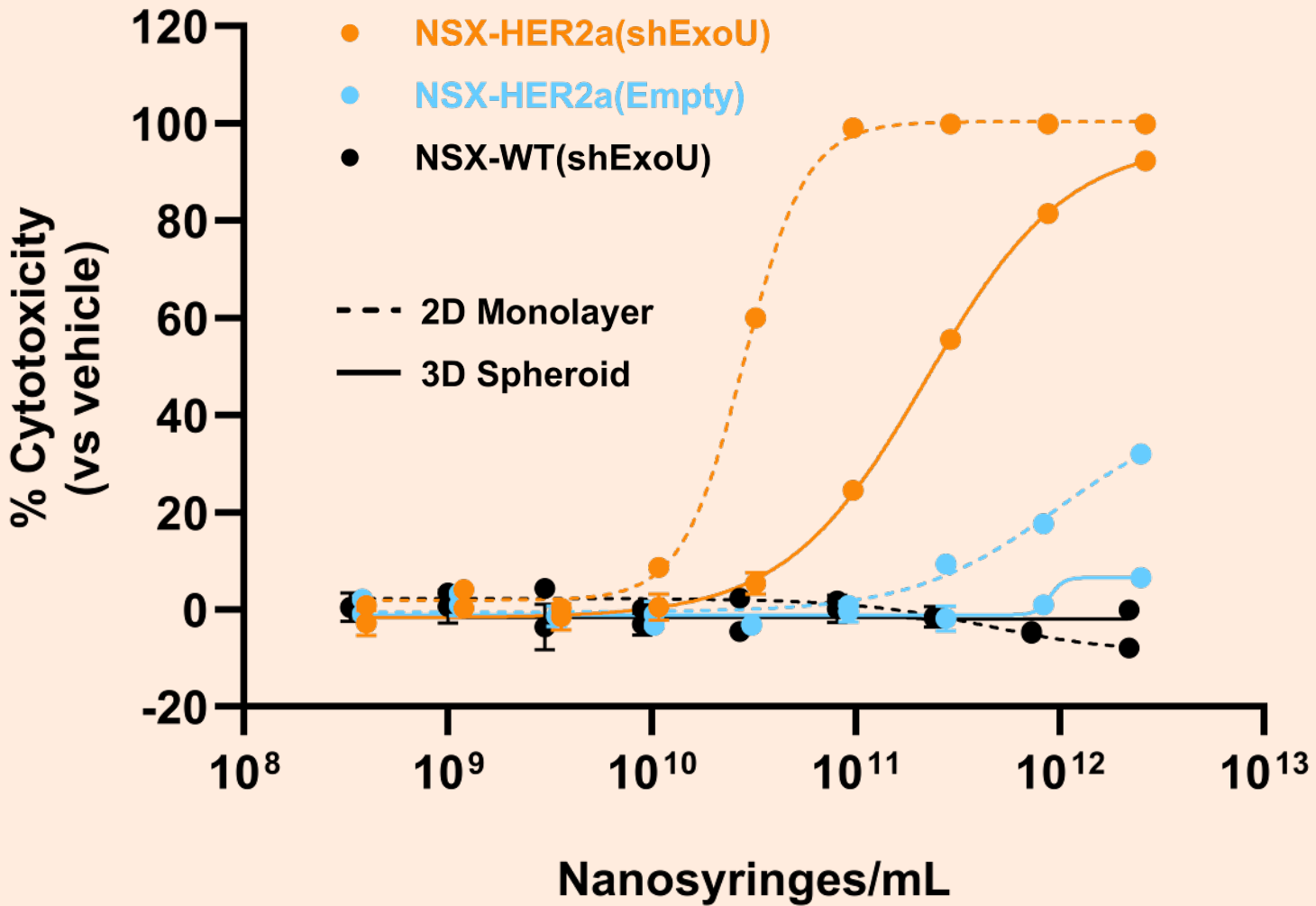


**6** Nanosyringes actively pierce the membrane deliver the 'API'



Nanosyringe potential and current development:  
*In vitro* validation with an oncology PoC

Demonstrated therapeutic proof-of-concept with a **potent** (nM), **fast-acting** (~24h), and **highly targeted** **Nanosyringe** delivering an intracellular (protein) cytotoxin supported by strong 2D and 3D *in vitro* data



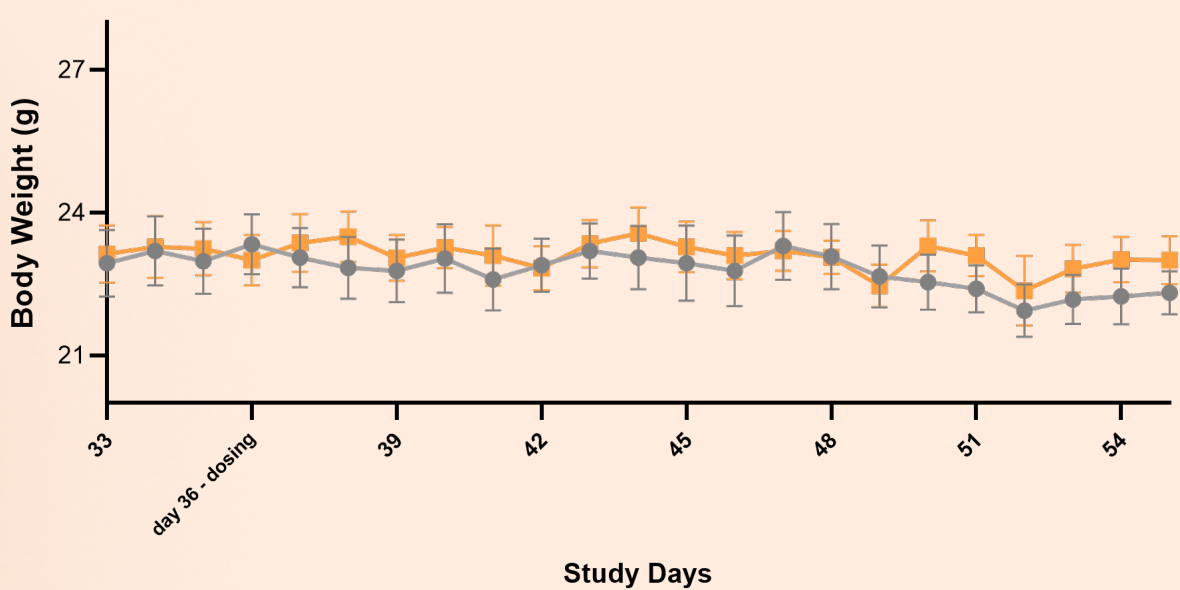
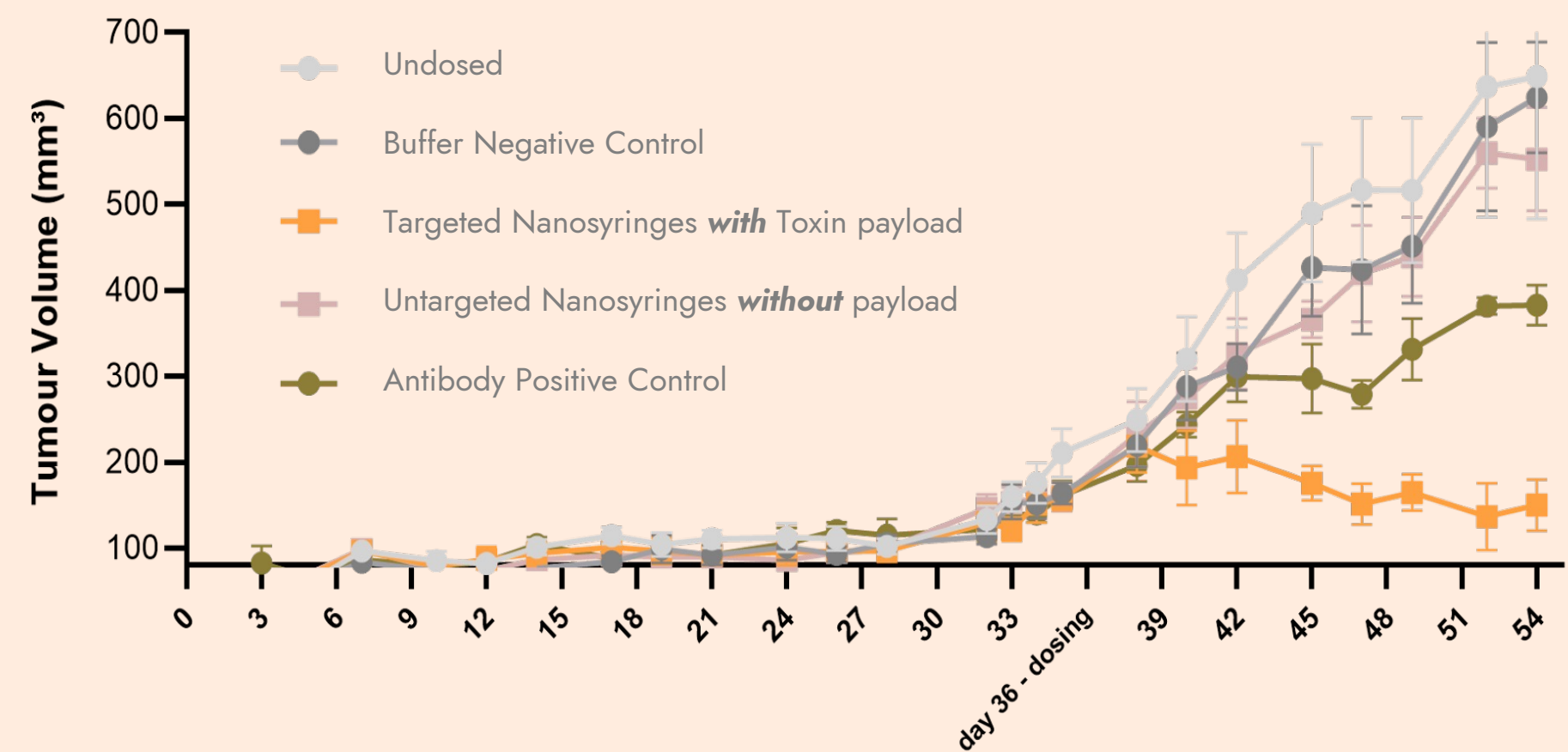
Sub-lethal doses of toxin-loaded Nanosyringes (2.5x10<sup>9</sup> Nanosyringes/ml) applied daily demonstrate an accumulated cytotoxic effect in spheroid models



# Nanosyringe potential and current development:

## In vivo validation with an oncology PoC

The same molecule exhibits **excellent tolerability** and **significant efficacy** in a rodent xenograft model, with no impact on animal body weight/behaviour, and observed **tumour regression**.

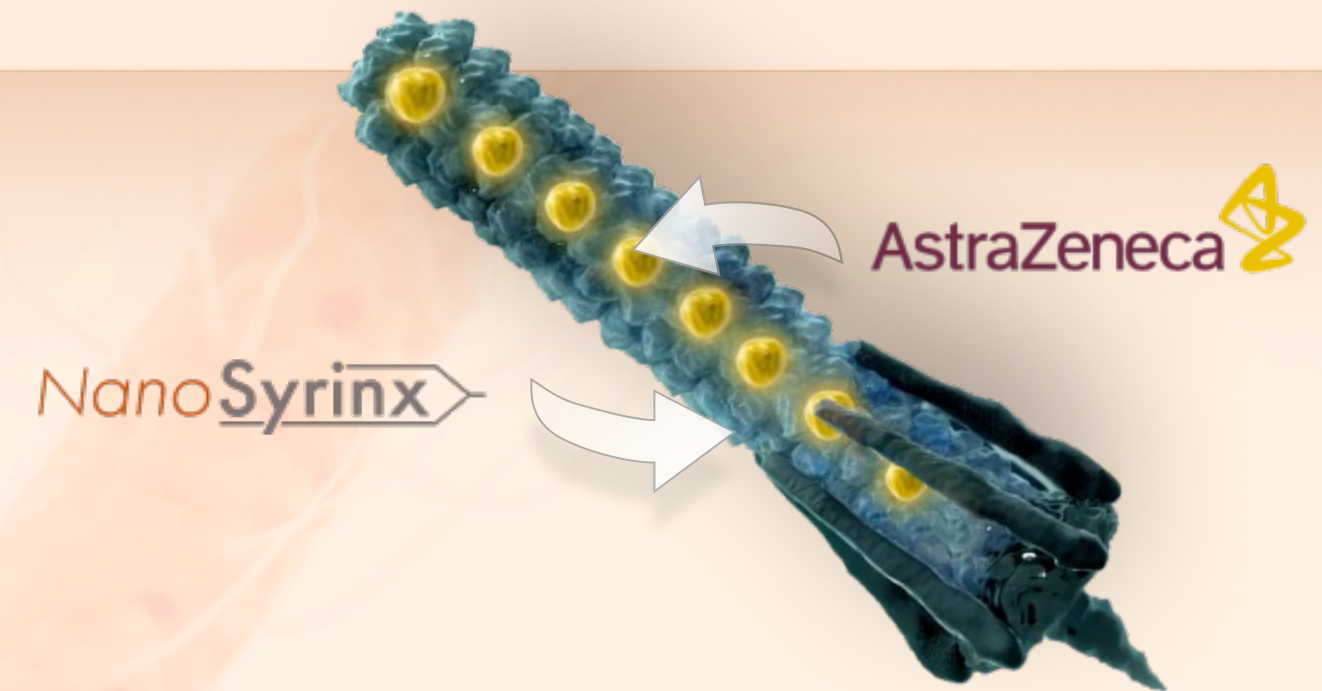


**Animals maintained bodyweight throughout dosing and exhibited no adverse clinical signs**



# 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.



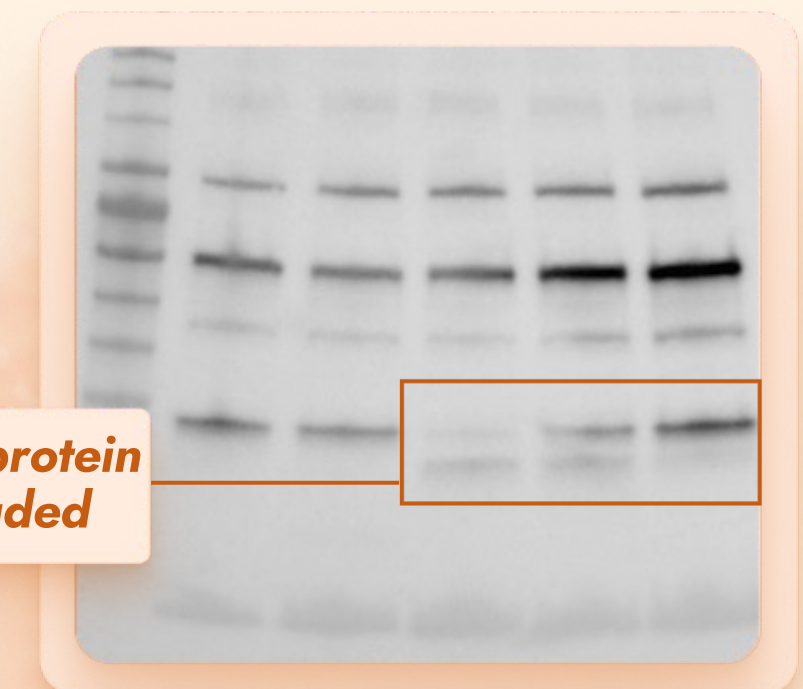
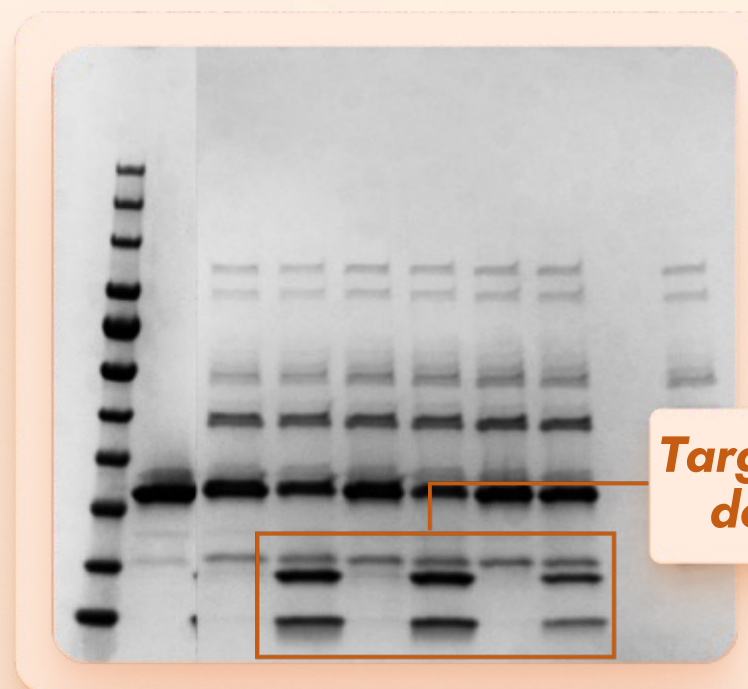
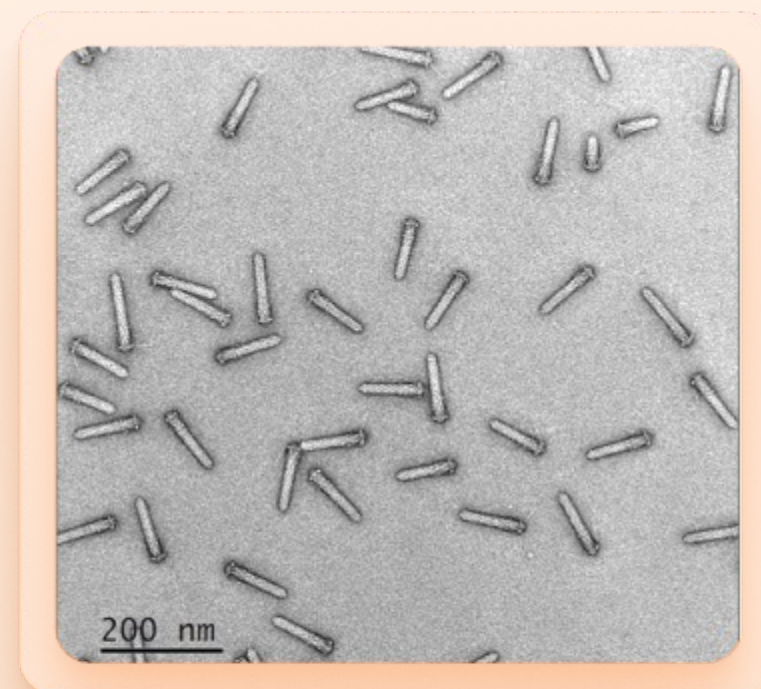
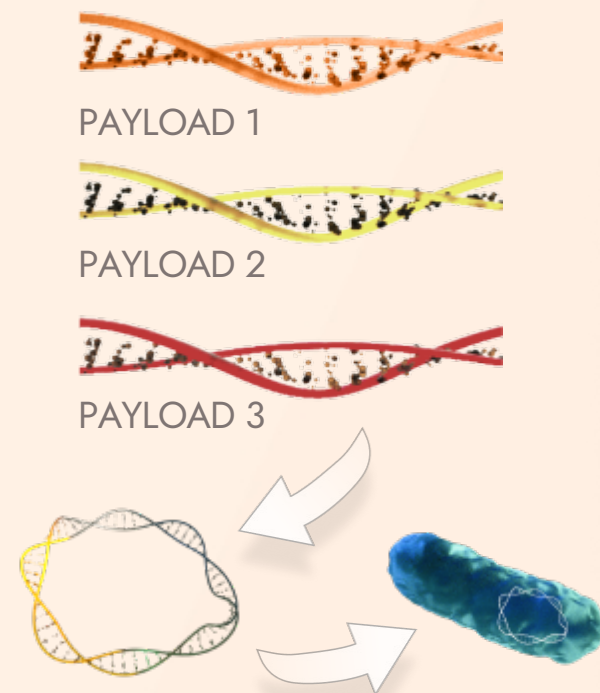
## Example workflow:

**1** Clone partner payload into proprietary genetic platform

**2** Confirm expression/loading/assembly

**3** Confirm packaged payload is functional

**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).

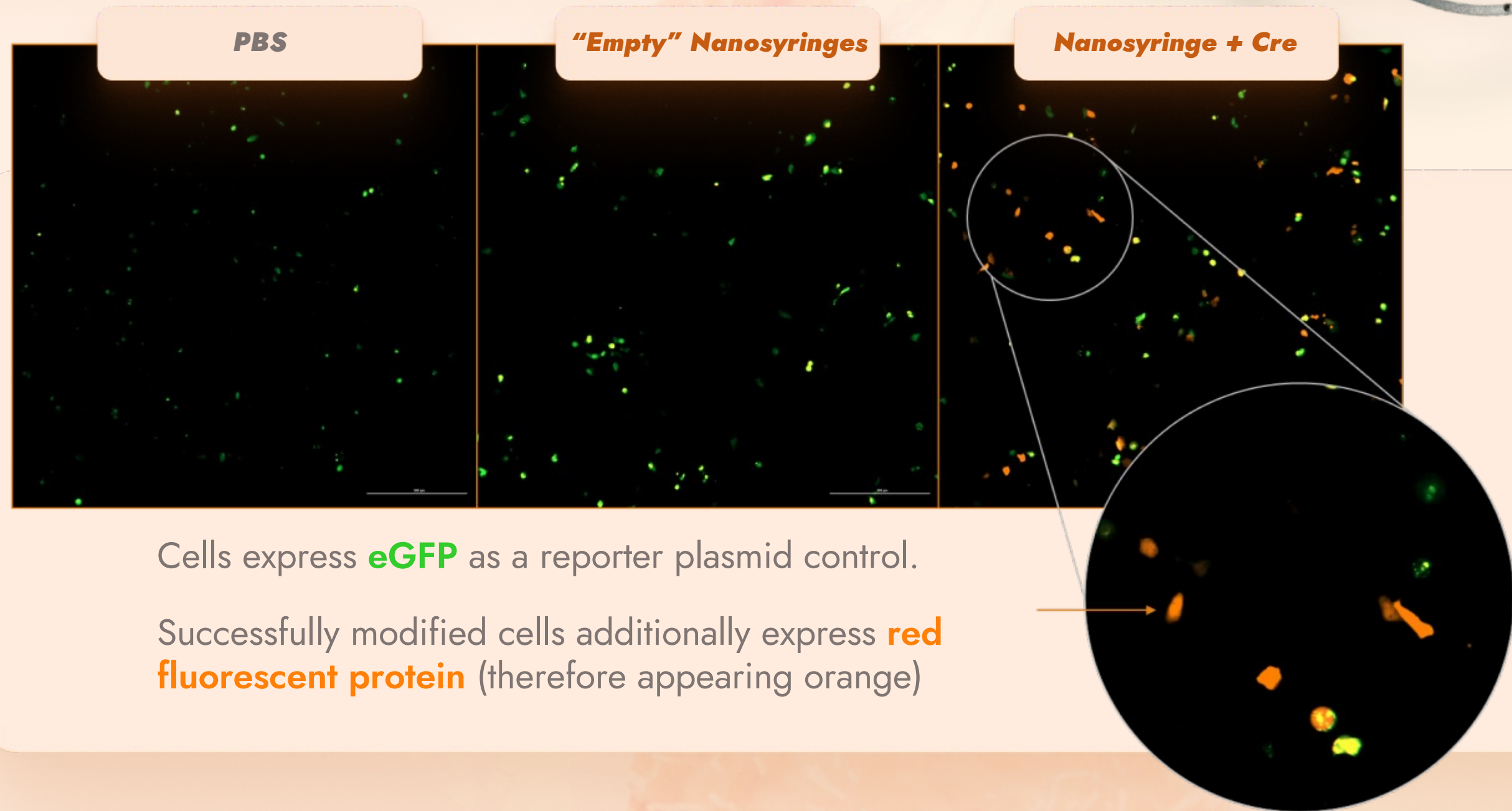


# A versatile delivery platform beyond Oncology

## Unprecedented access to the cell interior

Nanosyringes are able to **selectively deliver payloads capable of reaching intracellular compartments** (e.g nucleus) and modifying DNA opening up the potential for non-viral genetic engineering and similar approaches.

With further examples of DNA editing approaches appearing in the literature including Cas9 and Zn-finger containing proteins.

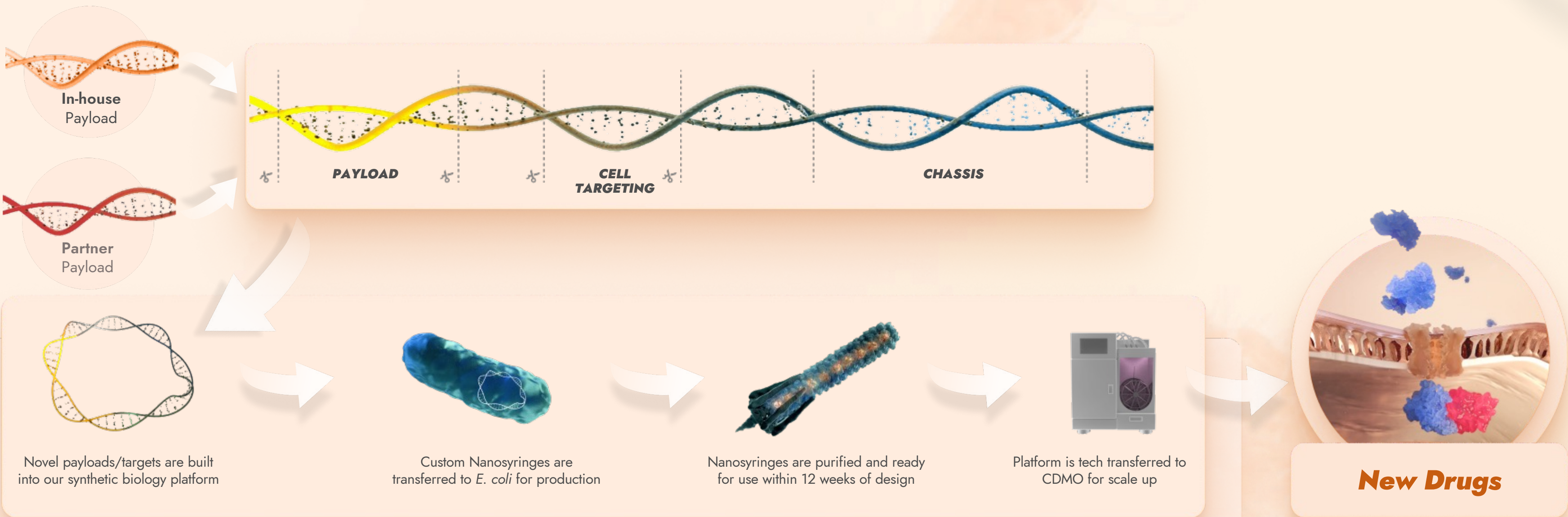




# NanoSyrinx Platform & Business Model

## Hybrid Approach:

We develop in-house programs for undruggable targets and engage in collaborative co-development and discovery with pharma/biotech to solve their biologic delivery challenges



**NanoSyrinx platform applied to targets**





# Want to learn more?

**[nanosyrinx.com](https://nanosyrinx.com)**

**[joe@nanosyrinx.com](mailto:joe@nanosyrinx.com)**

