

# GenoSafe

## **Analytical Services Expert**

for the evaluation of biological therapeutic  
products

<https://www.genosafe.com>



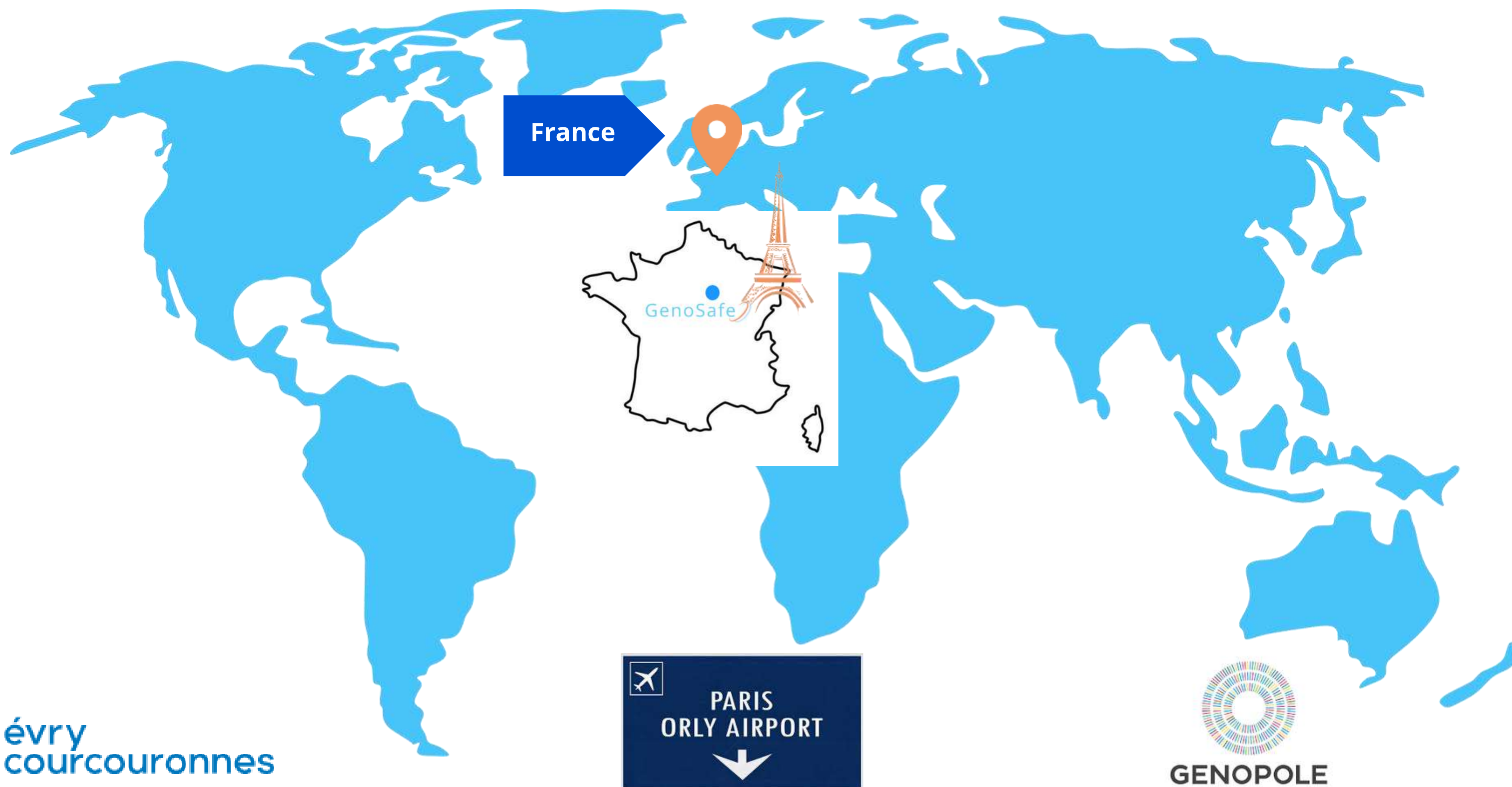
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The logo for GenoSafe, featuring the company name in a blue sans-serif font. To the right of the text is a stylized graphic consisting of two curved, overlapping lines, one orange and one light blue, forming a shape reminiscent of a DNA helix or a protective shield.



# GenoSafe Location



**Ideally Located Near Paris & Orly Airport**



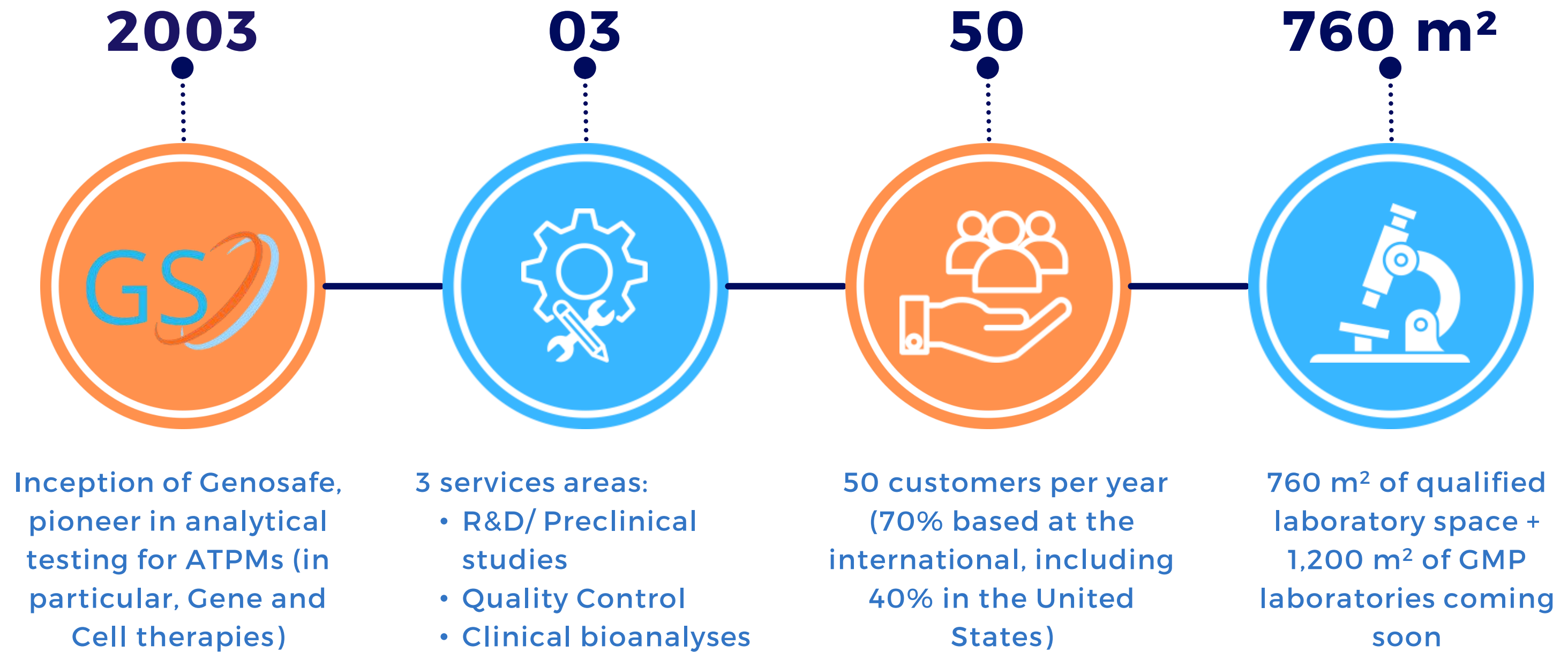
**Largest biocluster in France**



# An international client base



# GS Key figures



## **Preclinical studies**



GLP certified  
since **2008**

Involved in more  
than **40** preclinical  
studies overall

**30,000** samples  
analysed in 17  
years



# Key figures

## Quality control

More than **750**  
drug samples  
analysed each year

**15** new tests  
validated each year  
according to ICH Q2  
guidelines

An upcoming GMP  
facility expected in  
**2025**





# Key figures

## Clinical bioanalyses

Since 2015,  
involved in **50**  
clinical studies in **35**  
different indications

**10,000** clinical  
samples analyzed to  
date

**90** validated  
methods including  
15 generic methods





# Analytical services for innovative medicines

## Gene therapies, vaccines and other DNA/RNA based products

Viral vectors  
(AAV, Lentivirus...)

RNA based  
therapeutics

DNA based  
therapeutics

## Engineered cell therapies

CAR-T TCR-T CAR-NK

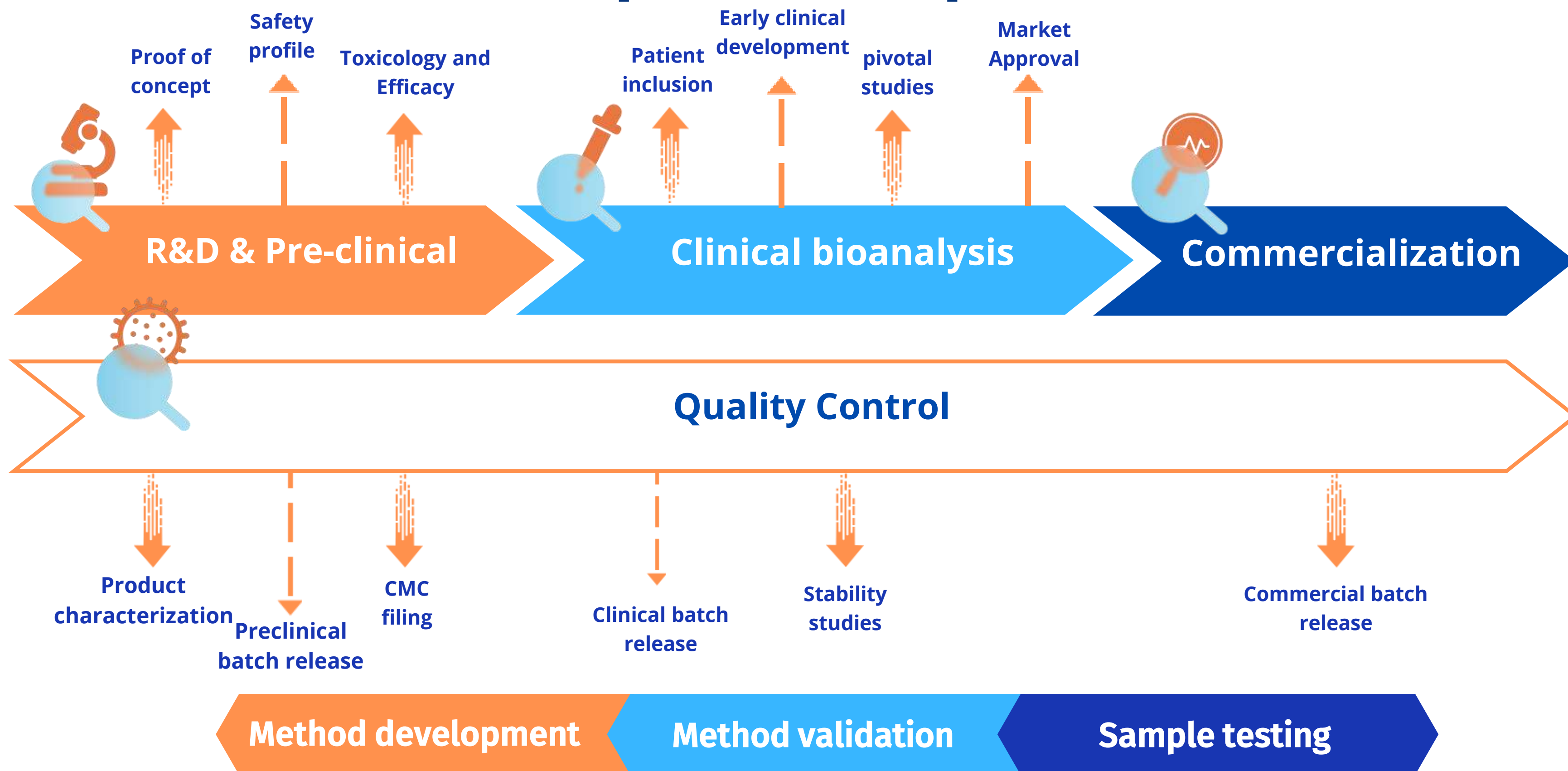
## Cell Therapies

Stem cell-derived therapeutics

iPS-derived therapeutics



# Supporting your drug candidate at each development step



# Preclinical evaluation and development

## Biodistribution and expression studies

- DNA extraction and purification steps optimized for more than 50 organs, tissues and fluids.
- Gene expression assays (mRNA and protein)

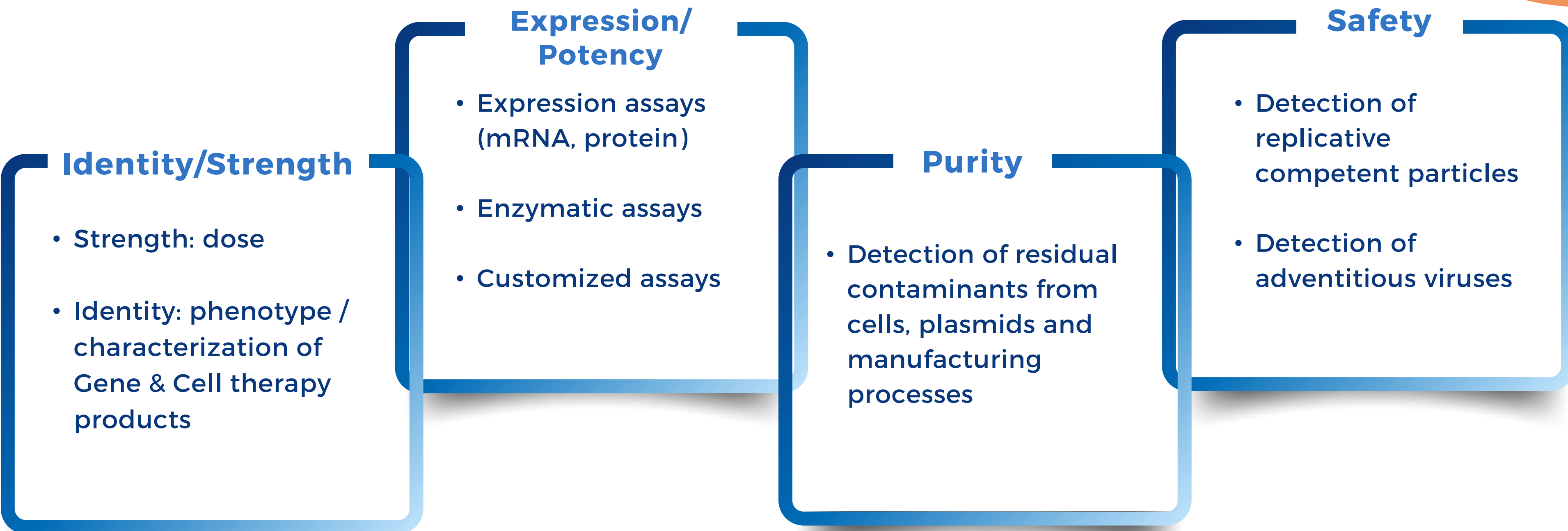
## Immunogenicity/ Biomarkers

- Humoral response (ADA, neutralizing factors)
- Cellular response (ELISpot, cytokines & chemokines)
- Detection and quantification of biomarkers





# Quality Control testing



Methods are developed and testing are performed in accordance with the European Pharmacopoeia and FDA guidelines. GenoSafe's labs are audited by customers to ensure GMP-compliant service



# Focus: QC for Gene Therapies

## Strength

### Viral Titration:

- Physical titration (p24, capsid)
- Viral Genome titration
- Infectious genome titration

## Safety

### Detection of replication-competent viruses:

- rcAAV
- RCL
- RCR

### Detection of adventitious viruses

## Purity

### Detection of specific contaminants from cells, plasmids and production-purification processes:

- VP1 VP2 VP3
- DNA: Plasmid, SF9, HEK
- AUC (full/empty capsids)
- DLS (Aggregation)

## Expression/ Potency

### Expression, enzymatic or customized potency assays:

- RT-qPCR/western-blot/ELISA/cell-based assays



# Focus: QC for Cell Therapies

## Identity

### Characterization of Cell therapy products:

- Flow cytometry analysis (e.g. CD45/CD19/CD4...)
- Biomarkers (cell differentiation markers by RT-qPCR)
- Viability (cell count)

## Potency/Efficacy

### Cellular response:

- ELISpot : interferon Gamma, evaluation of T-Cell responses
- ELISA, MSD multiplex : cytokine/chemokine measurement

### Cytotoxicity

## Purity

- Detection of residual DNA or protein

## Safety

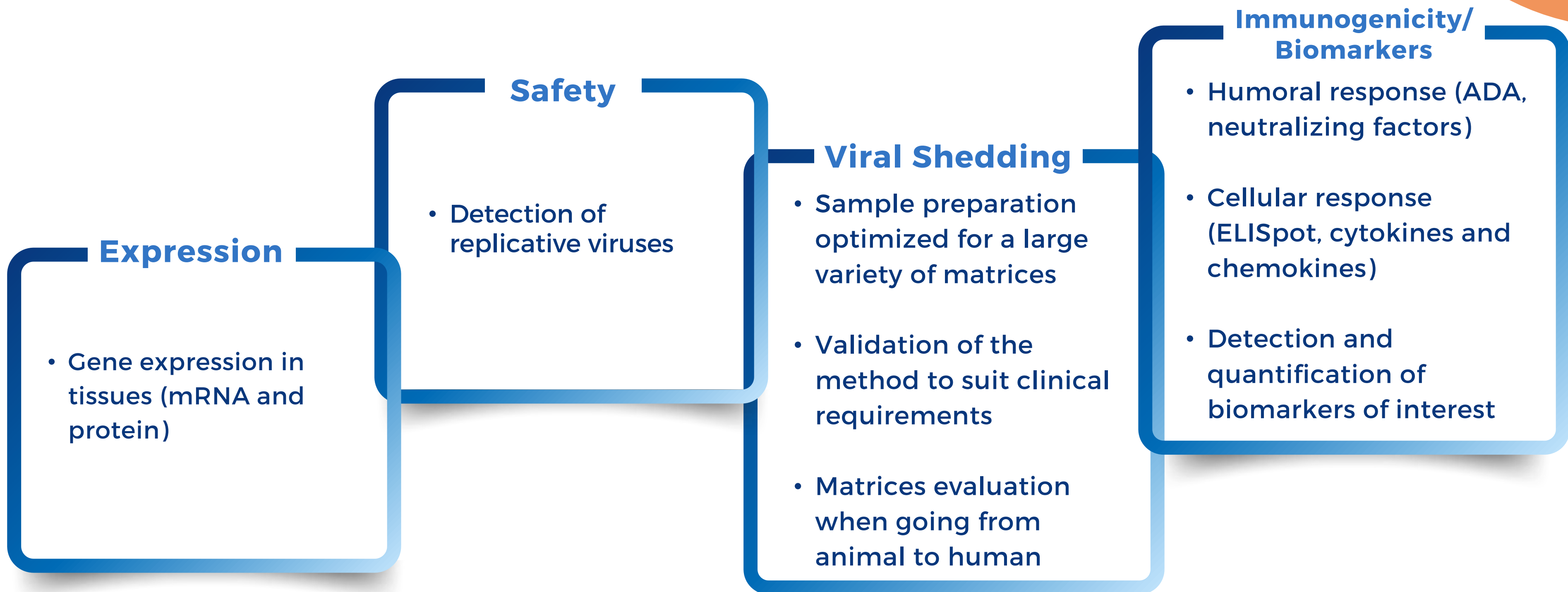
### Detection of replication-competent viruses:

- RCL or RCR on the vector and EoP
- rcAAV on the cell product and vector
- Vector Copy Number

### Detection of adventitious viruses



# Clinical sample analysis



# Focus on the immunogenicity assays

## Cellular response

- ELISpot : interferon gamma for the evaluation of T-Cell responses
- ELISA, MSD - multiplex : cytokine/chemokine measurement
- Cell phenotype by flow cytometry

## Humoral response

- Anti-drug antibody responses, detection / titration of binding IgG antibodies specific for AAV Capsids, IgG antibodies specific for transgenes (ELISA, ECLa (MSD platform))
- Detection / titration of neutralizing antibodies (cell-based)



# Equipment and platforms

- Spectrophotometry
- Flow cytometry
- Nucleic acid purification and extraction (manual and automated)
- PCR platforms (qPCR / RTqPCR / dPCR)
- Luminometry
- ELISA platform
- Automated nucleic acid extraction
- Meso Scale Discovery (MSD-ECLA)
- Western blot (traditional or capillary)
- ImmunoSpot (ELISpot / FluoroSpot)



# The expert team



**Alain Lamproye**

Chief Executive Officer



**Sabrina Triffault**

Study Director  
Preclinical and Clinical  
Testing



**Laurence Jeanson-Leh**

Study Director  
Quality Control



**Jens-Brice Marteau**

Study Director  
Quality Control



**William Lostal**

Study Director  
Preclinical and Clinical  
Testing



**Sarra Seninet**

Study Director  
Preclinical and Clinical  
Testing



**Agnès De Lacroix**

Study Director  
Quality Control

## Ongoing European collaborative projects and consortiums



### **CureCN (EU-H2020)**

Adeno-Associated Virus-Mediated Liver Gene Therapy for Crigler-Najjar Syndrome. Genosafe is responsible for the set-up of quality controls, biodistribution/ bioexpression and patient follow-up.

### **UshTher (EU-H2020)**

Clinical trial involving a gene therapy with dual AAV vectors for retinitis pigmentosa in patients with Usher syndrome type IB. Genosafe is responsible for the biodistribution, viral shedding and immunogenicity analysis.

### **ARDAT (EU-H2020)**

Consortium involving key players in the field of AAV gene therapy and aiming at "Accelerating Research & Development for Advanced Therapies"





## Contact Us



**CHRISTOPHE POQUET**  
Head of Business  
Development



**Phone**

+33 7 85 23 63 02



**Mail**

cpoquet@genosafe.com



**JEAN-MARIE TANTOT**  
Business Development  
Manager



**Phone**

+33 1 64 98 23 53



**Mail**

jmtantot@genosafe.com



**LINA SOUAGUI**  
Business Development  
Manager



**Phone**

+33 1 64 98 23 53



**Mail**

lsouagui@genosafe.com



**Website**

[www.genosafe.com](http://www.genosafe.com)



**Address**

1 rue de l'Internationale/ BP40064/ F-91002  
Evry-courcouronnes Cedex/France