

PHARMACEUTICAL VALUE CHAIN

DRUG DISCOVERY & DEVELOPMENT

CLINICAL TRIALS

COMMERCIALIZATION

Target

Lead

Pre-clinical

Phase I (first-in-human)

Phase IIa (proof-of-concept)

Phase IIb

Phase III

Filing

Launch

Companies and Activity



✓ Target validation with morpholinos

✓ Disease Models Generation

✓ Disease Models generation

✓ Toxicity assays

- Teratotox
- Neurotox
- Cardiotox
- Immunotox

✓ Efficacy assays

- CNS
- Cancer
- Metabolism
- Cardiovascular
- Rare diseases

✓ Disease Models generation

✓ Toxicity assays

- Teratotox
- Neurotox
- Cardiotox
- Immunotox

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- ✓ **Bioanalysis**
- ✓ Generic non-GLP methods for in vivo/in vitro ADMET screening

- ✓ **Bioanalysis**
- ✓ Development of non-GLP methods for in vivo/in vitro ADMET studies
- ✓ Preliminary PK/Tox

- ✓ **Bioanalysis**
- ✓ Development & Validation of bioanalytical methods in animal biological matrices
- ✓ PK&TK in animal species
- ✓ Metabolism
- ✓ Biomarkers
- ✓ Immunogenicity

- ✓ **Bioanalysis**
- ✓ Development and validation of bioanalytical methods in human biological matrices
- ✓ PK Clinical
- ✓ Biomarkers
- ✓ Immunogenicity

- ✓ **Bioanalysis**
- ✓ Clinical sample analysis
- ✓ Biomarkers
- ✓ Immunogenicity

- ✓ **Bioanalysis**
- ✓ Readjustment of bioanalytical methods
- ✓ Clinical sample analysis
- ✓ Biomarkers
- ✓ Immunogenicity
- ✓ Metabolism
- ✓ Long term toxicity

- ✓ **Bioanalysis**
- ✓ Clinical sample analysis
- ✓ Biomarkers
- ✓ Immunogenicity
- ✓ Metabolism
- ✓ New methods for reproductive-teratogenic studies
- ✓ Long term toxicity

- ✓ **Bioanalysis**
- ✓ Support to CTD elaboration

- ✓ **Bioanalysis**
- ✓ Line extensions
- ✓ Bioequivalences
- ✓ Post marketing clinical trials
- ✓ Immunogenicity

- ✓ **CMC**
- ✓ Analytical support to synthesis and HTS

- ✓ **CMC**
- ✓ API structural characterization
- ✓ API physico-chemical properties
- ✓ Development of analytical methods for API
- ✓ Analytical support to synthesis process
- ✓ Preliminary stability

- ✓ **CMC**
- ✓ API structural characterization
- ✓ Development & Qualification of analytical methods for API and first formulations
- ✓ Active ingredient standardization
- ✓ Preliminary stability
- ✓ Stress testing

- ✓ **CMC**
- ✓ Characterization of active ingredient
- ✓ Impurity profile
- ✓ Analytical support to formulation development
- ✓ Stability
- ✓ Microbiology

- ✓ **CMC**
- ✓ Characterization of active ingredient
- ✓ Analytical support to formulation development
- ✓ Stability
- ✓ Microbiology

- ✓ **CMC**
- ✓ Characterization of active ingredient
- ✓ Analytical support to development final formulation
- ✓ Full validation control methods
- ✓ Analytical support to scale-up of manufacturing process
- ✓ Stability ICH
- ✓ QbD approaches
- ✓ In-use stability

- ✓ **CMC**
- ✓ Full validation control methods
- ✓ Analytical support to manufacturing process / critical points
- ✓ Validation of manufacturing process
- ✓ Stability ICH
- ✓ NIR & RAMAN for identification of raw materials
- ✓ QbD approaches
- ✓ PAT in process control
- ✓ Elemental impurity control

- ✓ **CMC**
- ✓ Support to CTD elaboration

- ✓ **CMC**
- ✓ Quality Control
- ✓ Batch testing
- ✓ Batch release
- ✓ Raw material analysis
- ✓ On-going stability
- ✓ NIR & RAMAN for identification of raw materials

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 <ul style="list-style-type: none"> ✓ Mechanism of action characterization 	 <ul style="list-style-type: none"> ✓ Efficacy <i>in vitro</i>, <i>ex vivo</i> and <i>in vivo</i> studies ✓ Proof of concept 	 <ul style="list-style-type: none"> ✓ All techniques to preclinical studies including characterization of responses in human cells 						
 <ul style="list-style-type: none"> ✓ Kinetic Profiling to unveil unexpected therapeutic targets ✓ Assay Development to address novel modes of action and challenging targets ✓ HTS campaigns 	 <ul style="list-style-type: none"> ✓ Determine accurate kinetic values to identify slow binding compounds or compounds that induce conformational changes. ✓ Identify irreversible compounds and determine accurate kinact/KI values. ✓ Generate SAR and SKR for reversible and irreversible compounds. ✓ Understand the MoA to optimize efficient compounds. 	 <ul style="list-style-type: none"> ✓ Determine kinetic selectivity to asses physiologically relevant off-target risks. 						

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Phase IIb

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Companies and Activity

Target	Lead	Pre-clinical	Phase I (first-in-human)	Phase IIa (proof-of concept)	Phase IIb	Phase III	Filing	Launch
		 <ul style="list-style-type: none"> ✓ Pre-formulation studies ✓ Formulation screening ✓ Development of formulation for pre-clinical studies ✓ Parenteral process development: Solutions for injection and Lyophilized products ✓ Early analytics ✓ Early stability ✓ Project management 	 <ul style="list-style-type: none"> ✓ Development of formulations for Phase I ✓ Analytical development ✓ Quality control strategy ✓ Stability studies ✓ Clinical supply for phase I ✓ QP release ✓ Regulatory support ✓ Project management 	 <ul style="list-style-type: none"> ✓ Development of formulations for Phase IIa ✓ Analytical development ✓ Quality control strategy ✓ Stability studies ✓ Clinical supply for phase IIa ✓ QP release ✓ Regulatory support ✓ Project management 	 <ul style="list-style-type: none"> ✓ Development of formulations for Phase IIb ✓ Analytical development ✓ Quality control strategy ✓ Stability studies ✓ Clinical supply for phase IIb ✓ QP release ✓ Regulatory support ✓ Project management 	 <ul style="list-style-type: none"> ✓ Development of formulations for Phase III ✓ Analytical development ✓ Quality control strategy ✓ Stability studies ✓ Clinical supply for phase III ✓ QP release ✓ Regulatory support ✓ Project management 	 <ul style="list-style-type: none"> ✓ Development of formulations for application of marketing authorization ✓ Scale-up ✓ Technology transfer ✓ Process validation ✓ Exhibit batches manufacturing ✓ Stability studies ✓ Regulatory support ✓ Project management 	 <ul style="list-style-type: none"> ✓ Manufacturing of commercial batches ✓ Life cycle management
		 <ul style="list-style-type: none"> ✓ Business Plan ✓ Fundraising ✓ Market studies ✓ Business Development 	 <ul style="list-style-type: none"> ✓ Business Plan ✓ Fundraising ✓ Market studies ✓ Business Development 	 <ul style="list-style-type: none"> ✓ Business Plan ✓ Fundraising ✓ Market studies ✓ Business Development 	 <ul style="list-style-type: none"> ✓ Business Plan ✓ Fundraising ✓ Market studies ✓ Business Development 	 <ul style="list-style-type: none"> ✓ Market Access ✓ Business Plan ✓ Fundraising ✓ Market studies ✓ Business Development 	 <ul style="list-style-type: none"> ✓ Market Access ✓ Market studies ✓ Business Development 	 <ul style="list-style-type: none"> ✓ Market Access ✓ Market studies ✓ Business Development

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Drug Discovery & Development			Clinical Trials				Commercialization	
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			 <ul style="list-style-type: none"> ✓ Clinical development planning ✓ Regulatory affairs ✓ Medical informatics ✓ Global clinical operations ✓ Study and site coordination ✓ Patient recruitment services ✓ Clinical data management ✓ Biostatistics ✓ Pharmacovigilance / safety ✓ Medical writing ✓ Quality assurance ✓ Clinical service solutions 	 <ul style="list-style-type: none"> ✓ Clinical development planning ✓ Regulatory affairs ✓ Medical informatics ✓ Global clinical operations ✓ Study and site coordination ✓ Patient recruitment services ✓ Clinical data management ✓ Biostatistics ✓ Pharmacovigilance / safety ✓ Medical writing ✓ Quality assurance ✓ Clinical service solutions 	 <ul style="list-style-type: none"> ✓ Clinical development planning ✓ Regulatory affairs ✓ Medical informatics ✓ Global clinical operations ✓ Study and site coordination ✓ Patient recruitment services ✓ Clinical data management ✓ Biostatistics ✓ Pharmacovigilance / safety ✓ Medical writing ✓ Quality assurance ✓ Clinical service solutions 	 <ul style="list-style-type: none"> ✓ Clinical development planning ✓ Regulatory affairs ✓ Medical informatics ✓ Global clinical operations ✓ Study and site coordination ✓ Patient recruitment services ✓ Clinical data management ✓ Biostatistics ✓ Pharmacovigilance / safety ✓ Medical writing ✓ Quality assurance ✓ Clinical service solutions 	 <ul style="list-style-type: none"> ✓ Clinical development planning ✓ Regulatory affairs ✓ Medical informatics ✓ Global clinical operations ✓ Study and site coordination ✓ Patient recruitment services ✓ Clinical data management ✓ Biostatistics ✓ Pharmacovigilance / safety ✓ Medical writing ✓ Quality assurance ✓ Clinical service solutions 	 <ul style="list-style-type: none"> ✓ Clinical development planning ✓ Regulatory affairs ✓ Medical informatics ✓ Global clinical operations ✓ Study and site coordination ✓ Patient recruitment services ✓ Clinical data management ✓ Biostatistics ✓ Pharmacovigilance / safety ✓ Medical writing ✓ Quality assurance ✓ Clinical service solutions
	 <ul style="list-style-type: none"> ✓ Bioprocess development ✓ Lentiviral vectors ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ Lentiviral vectors manufacture for PC Tox. ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ GMP manufacture of LV vectors ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ GMP manufacture of LV vectors ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ GMP manufacture of LV vectors ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ GMP manufacture of LV vectors ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ GMP manufacture of LV vectors ✓ Lentisoma technology collaboration 	 <ul style="list-style-type: none"> ✓ GMP manufacture of LV vectors ✓ Lentisoma technology collaboration