

# Leveraging Advanced Partnering Data



September 24, 2024

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**Inpart Summit**  
User Group Meeting

**Echo Zhang**  
VP Product Strategy | **Inpart**

**Rana Houry**  
Product Manager | **Inpart**



# The most important thing about data is that it is:

- A Accurate
- B Organized
- C Integrated
- D Actionable
- E All of the above

# Inpart Deal

## Search & evaluation

Finding the right partners faster

## Business development

Managing opportunities

## Alliance management

Driving alliances towards success

## Due diligence

Leveraging compliance best practices



## Centralized data & intelligent integrations

Databases: Evaluate Pharma, Inpart Data

Everyday tools: Power BI, Microsoft Outlook

Events: BIO Connector, partnering conferences



## Real-time collaborations & reporting

Improve team engagement and alignment

Overview on partnering efforts and pipeline



## Data integrity & compliance

Safety and compliance built into workflows

# Having good data is challenging – pain points



Entering data is hard and the volume is high



Keeping data from multiple sources up to date is time-consuming



Difficult to democratize data



Challenging to pull reports

# Find relevant internal and external data

Instantly find internal and external data on a company for a 360-view

**Sarepta Therapeutics**

Type: Pharma, Biotech, ... Agreements: CDA

Opportunities 2 | Contacts 1 | Meetings 3 | Attachments 0 | Agreements 1

**Opportunities 2**

Search: Search with keyword | Type/Stage: All | Status: All | More filters

Title	Type / Stage	Initiative	Status
SRP-6004	To be defined Screening	NASH	Active
SRP-5051	In-Licensing Full Assessment	Rare disease	Active

**Tags**

Search for tags...

**Notes**

Write notes about this company

**Details**

United States  
Cambridge  
215 First Street  
<https://www.sarepta.com>

**Overview**

Former: No data

**Summary**

Sarepta is a global biotechnology company on an urgent engineer precision genetic medicine to reclaim futures impacted or cut short by rare diseases.

**Description**

Sarepta is a global biotechnology company on an urgent engineer precision genetic medicine for rare diseases that lives and cut futures short. We're ushering in a new era of development with the goal of driving efficiencies, including the time from lab to patient and building the world's largest therapy manufacturing capacity. We're collaborating with networks and payers, rethinking pricing models for revolutionary treatments in development. We are in a daily race to transform understanding into genetic medicine. Because every day opportunity to save lives stolen by rare disease.

**Related Companies**

**Company Financials**

Number of employees: >1001

Market capitalization (m\$): 12.8

**Sarepta Therapeutics**

From Evaluate · Last updated May 11, 2023

Listed company

USA

Massachusetts · Cambridge

View in evaluate

**R&D projects 45**

By Development phase

Phase	Count
Filed	1
Phase II	5
Phase III	2
Pre-clinical	21
Research proj...	16

**Deals**

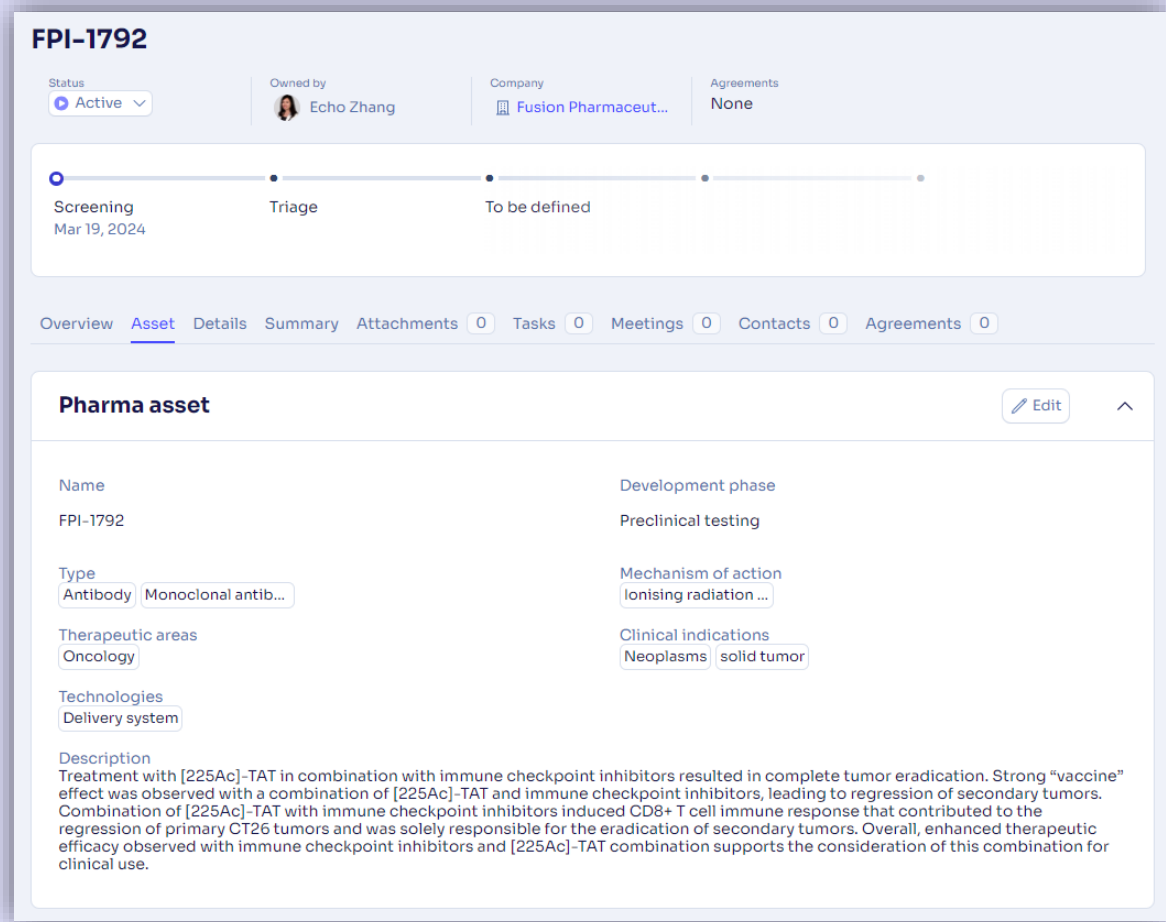
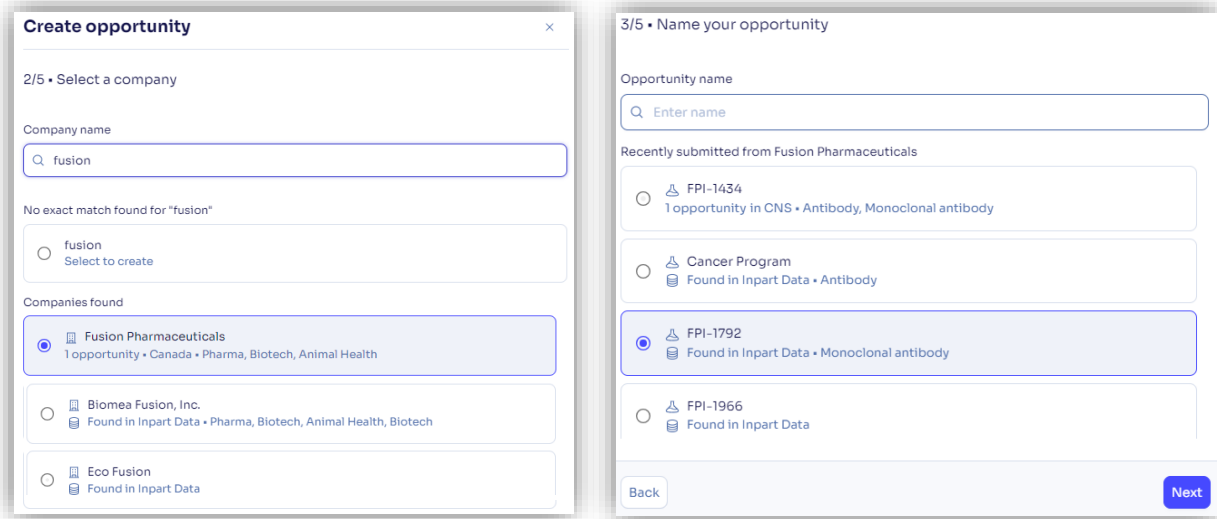
**Deals distributions 38**

By Deal Value

Deal Value	Count
< 100 m\$	7
100-499 m\$	10
500-999 m\$	12
> 1000 m\$	9

# Reduce manual data entry

Simplify opportunity creation by choosing company and asset profiles to use directly from Inpart's curated database



# Evaluate your opportunities

Leverage external data to facilitate evaluations of your opportunities

The screenshot displays a software interface for managing pharmaceutical assets. The main view is for asset **SRP-5051**, which is in the **Phase 2** development stage. A progress bar at the top shows the asset's lifecycle from **Screening** (Oct 3, 2023) through **Triage** (Oct 3, 2023), **Confidential Evaluation** (Nov 13, 2023), **Full Assessment** (Mar 14, 2024), **Final Negotiation**, **Sign and Close**, and **Closed**. The interface includes a sidebar with navigation icons and a top navigation bar with tabs for **Overview**, **Asset**, **Details**, **Summary**, **Attachments** (2), **Tasks** (5), **Meetings** (1), **Contacts** (1), and **Agreements** (1).

The **Pharma asset** section provides the following details:

- Name:** SRP-5051
- Development phase:** Phase 2
- Type:** Nucleotide and d... (tags: Oligonucleotide, Antisense, RNA, RNA)
- Mechanism of action:** Dystrophin expre...
- Therapeutic areas:** Rare Disease
- Clinical indications:** Muscular Dystrop...
- Technologies:** -

The **Description** states: "SRP-5051 is an investigational agent using Sarepta's PPMO chemistry and exon-skipping technology to skip exon 51 of the dystrophin gene. SRP-5051 is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally shortened, functional dystrophin protein. PPMO is Sarepta's next-generation chemistry platform designed around a proprietary cell-penetrating peptide conjugated to the PMO backbone, with the goal of increasing tissue penetration, increasing exon skipping, and significantly increasing dystrophin production. Around 13% of DMD patients have mutations that make them amenable to skipping exon 51. If successful, the PPMO offers the potential for improved efficacy and less frequent dosing for patients."

The **Overview** panel on the right provides a **Detailed Pipeline in Evaluate Pharma** with the following information:

- Therapeutic category:** Musculoskeletal
- Therapeutic subcategory:** Other musculoskeletal agents
- Indication summary table:**

Indication	Dev. phase	Status
Duchenne muscular dystrophy	Phase II	Active
Becker muscular dystrophy	Research pro...	Abandoned
Pompe's disease	Research pro...	Abandoned
Progeria/Hutchinson-Gilford sy...	Research pro...	Abandoned

- Proprietary level:** New molecular entity
- Technological category:** Biotechnology
- Technology:** DNA & RNA therapeutics
- Mechanism of action:** Dystrophin stimulant
- Annual sales (m\$):** No data
- Patent expiry date:** No data
- Comment:** Utilises proprietary phosphorodiamidate morpholino oligomer (PPMO) technology.

# Evaluate your opportunities with Evaluate Pharma

Inpart's algorithms automatically match your opportunities in Inpart Deal to profiles in Evaluate Pharma, providing in-depth data on companies and assets and the context you need to make the right decisions faster



Empower your teams to make data-driven decisions



Save time by aggregating your partnering information with external data

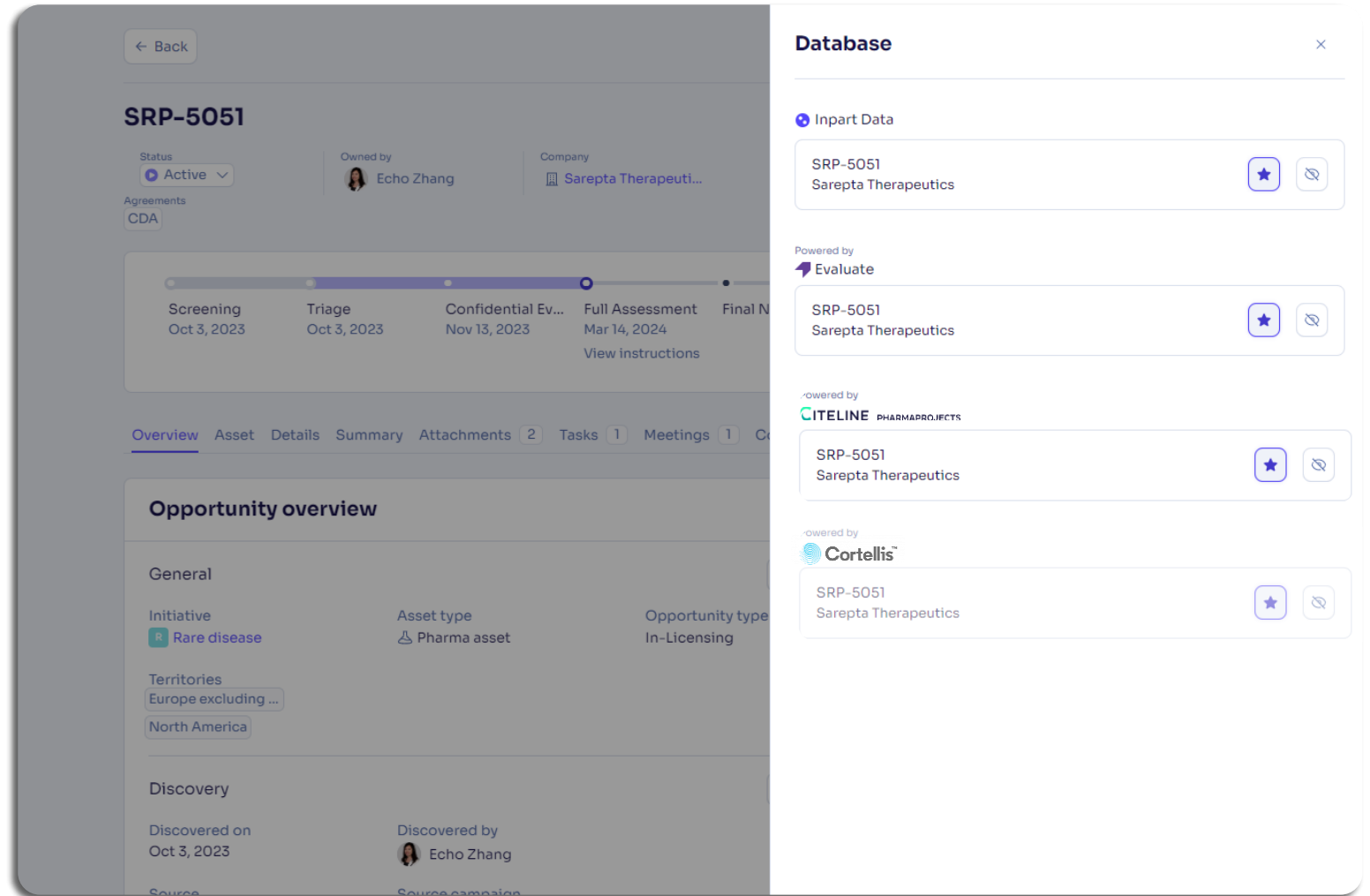
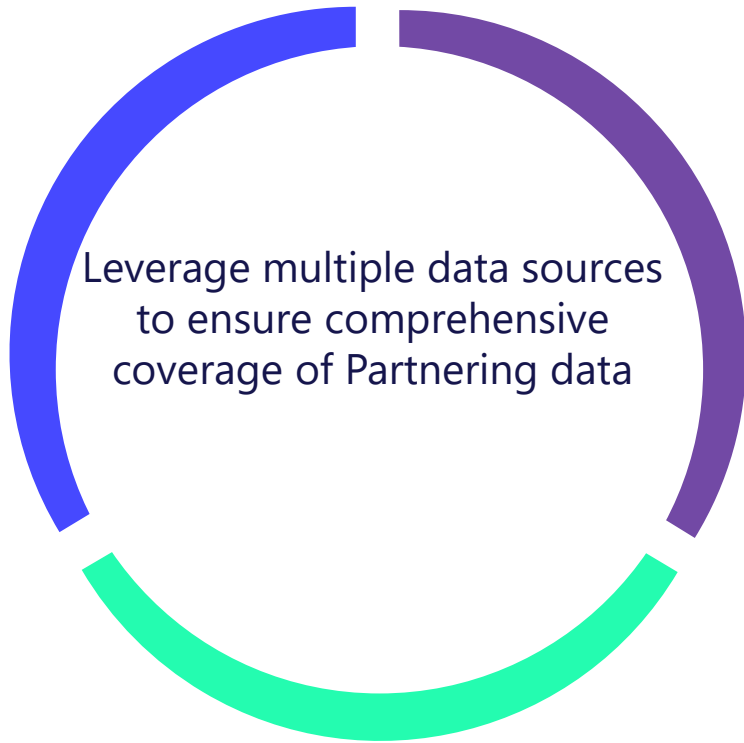


Access up-to-date data directly from the linked opportunities

The screenshot displays the Evaluate Pharma interface, a Norstella company. The main view is for 'AMONDYS 45', which is an active asset owned by Melissa Scott at Sarepta Therapeutics. A progress bar shows the stages: Identification (Oct 27, 2023), Triage (Nov 16, 2023), Confidential Ev... (Jan 17, 2024), and Due Diligence. Below this, there are tabs for Overview, Asset, Details, Summary, Attachments (1), Tasks (7), and Meetings. The 'Opportunity overview' section includes details for Initiative (Alzheimer), Asset type (Pharma asset), Opportunity type (In-Licensing), Territories (No geographical scope defin...), and Territories details (No territory details added). A sidebar on the right provides a detailed overview of 'Amondys 45', including search filters for 'casimersen' and 'Sarepta Therapeutics', and a section for 'Overview' with fields for Proprietary status (New molecular entity) and Technological category.



# More data sources for deeper evaluation



**SRP-5051**

Status: Active | Owned by: Echo Zhang | Company: Sarepta Therapeuti...

Agreements: CDA

Screening: Oct 3, 2023 | Triage: Oct 3, 2023 | Confidential Ev...: Nov 13, 2023 | Full Assessment: Mar 14, 2024 | Final N...

Overview | Asset | Details | Summary | Attachments (2) | Tasks (1) | Meetings (1) | C...

### Opportunity overview

**General**

Initiative: Rare disease | Asset type: Pharma asset | Opportunity type: In-Licensing

Territories: Europe excluding ... | North America

**Discovery**

Discovered on: Oct 3, 2023 | Discovered by: Echo Zhang

Source: | Source campaign:

### Database

Inpart Data

- SRP-5051 Sarepta Therapeutics

Powered by Evaluate

- SRP-5051 Sarepta Therapeutics

Powered by CITELINE PHARMAPROJECTS

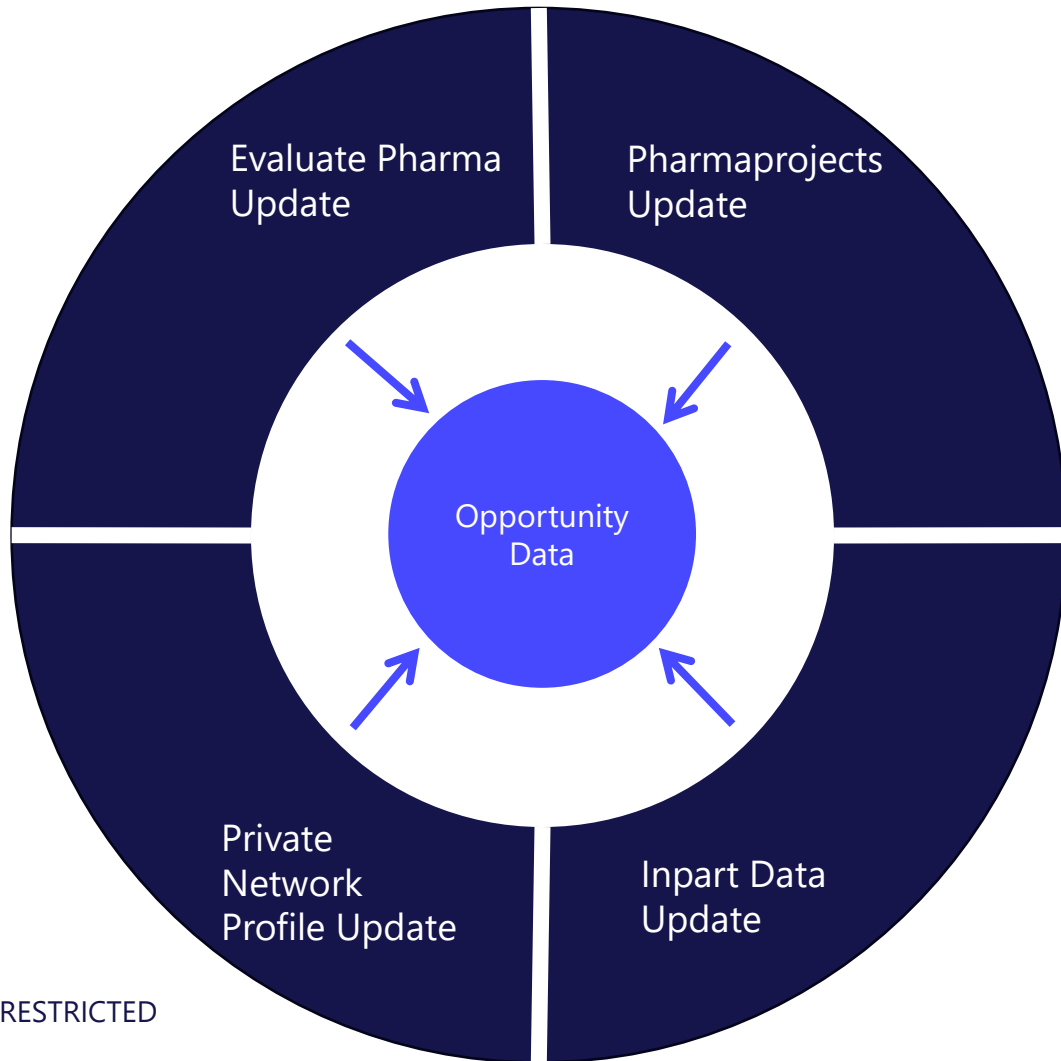
- SRP-5051 Sarepta Therapeutics

Powered by Cortellis™

- SRP-5051 Sarepta Therapeutics

# Updating Data

Ensure that your data is up to date through our multiple data integrations and proactively engage with nurtured companies

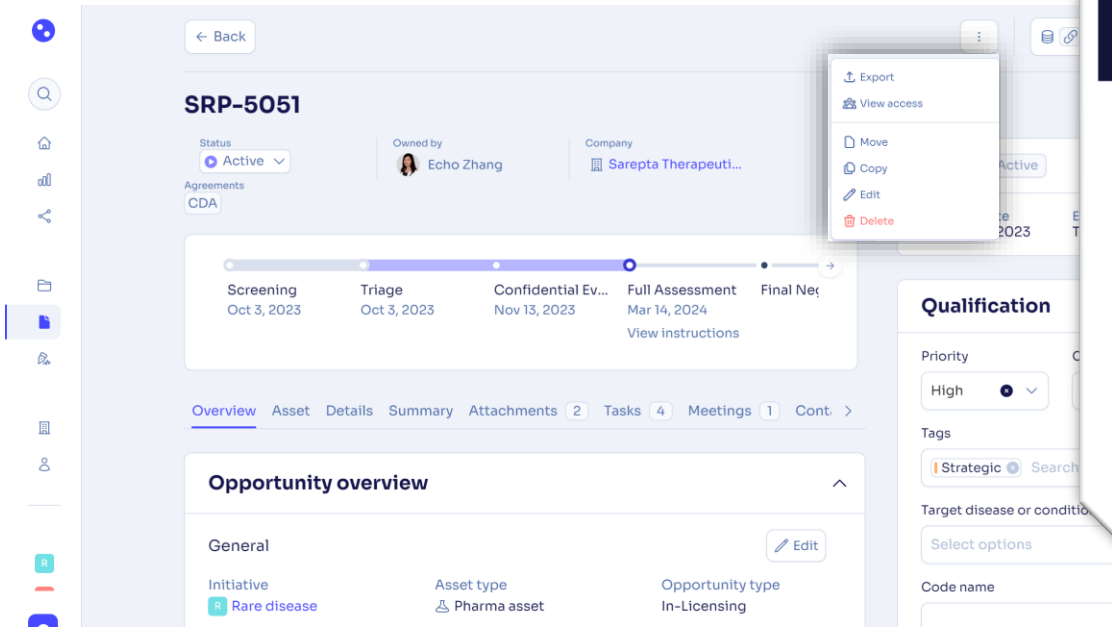


RESTRICTED

The screenshot shows a user interface for an 'Activity stream'. At the top, there's a title 'Activity stream' with a close button. Below the title, there's a section for 'Upcoming' events. The first event is 'Projected Phase 2 Announcement due on Aug 12, 2024', powered by 'BIOMEDTRACKER' (CITELINE COMMERCIAL). There is a '+ Add Reminder' button next to it. Below the upcoming events, there's a section for 'Earlier' activities. The first activity is 'Pre CDA Eval due on Oct 31, 2023'. The second activity is 'Approval for Conf Assessment due on Oct 27, 2023'. Below that, there's a partial view of another activity: 'Oct 18, 2023 • Atsumi Fujigasaki' with the text 'Opportunity type updated to In-licensing'.

# Reporting Automation

Generate presentation-ready reports from guaranteed high quality data to help take data-driven decisions



**SRP-5051**

Status: Active

Owned by: Echo Zhang

Company: Sarepta Therapeuti...

Agreements: CDA

Screening: Oct 3, 2023

Triage: Oct 3, 2023

Confidential Ev...: Nov 13, 2023

Full Assessment: Mar 14, 2024

Final Nex...: [Date]

View instructions

Overview | Asset | Details | Summary | Attachments (2) | Tasks (4) | Meetings (1) | Cont. >

### Opportunity overview

General

Initiative: Rare disease

Asset type: Pharma asset

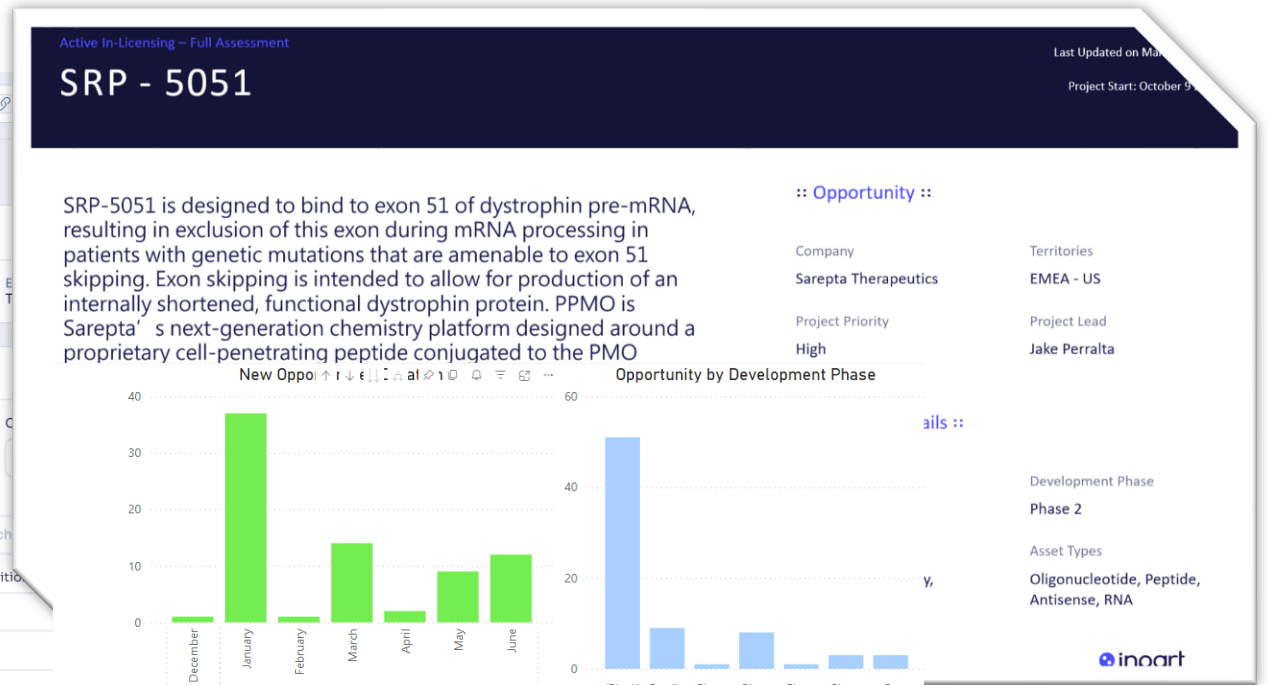
Opportunity type: In-Licensing

Qualification: High

Tags: Strategic

Target disease or condition: [Select options]

Code name: [Input field]



Active In-Licensing - Full Assessment

## SRP - 5051

Last Updated on Mar 14, 2024

Project Start: October 9, 2023

**SRP-5051** is designed to bind to exon 51 of dystrophin pre-mRNA, resulting in exclusion of this exon during mRNA processing in patients with genetic mutations that are amenable to exon 51 skipping. Exon skipping is intended to allow for production of an internally shortened, functional dystrophin protein. PPMO is Sarepta's next-generation chemistry platform designed around a proprietary cell-penetrating peptide conjugated to the PMO

**Opportunity**

Company	Sarepta Therapeutics	Territories	EMEA - US
Project Priority	High	Project Lead	Jake Peralta

**Opportunity by Development Phase**

Development Phase	Count
(Blank)	50
Precli... testing	10
Phase 0	2
Phase 1	10
Phase 2	2
Phase 3	2
On Market	2

**By Country**

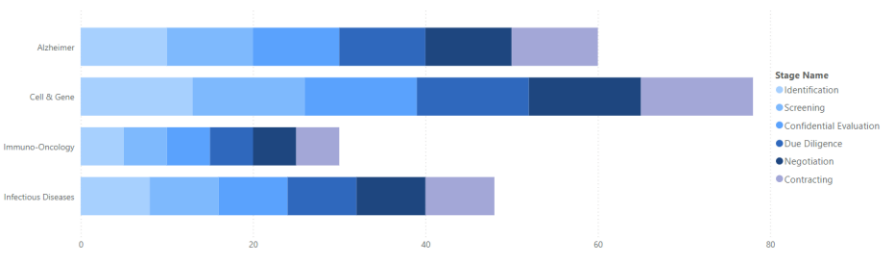
Country	Count	Percentage
United States	41	53.95%
United Kingdom	22	28.95%
Japan	5	6.58%
South Korea	1	1.32%
Denmark	1	1.32%
France	1	1.32%
Netherlands	1	1.32%
Taiwan	1	1.32%

**Stale Opportunities**

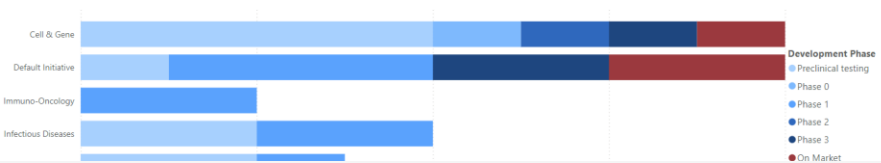
Month	Count
January	1
February 2023	5
March	10

inoart

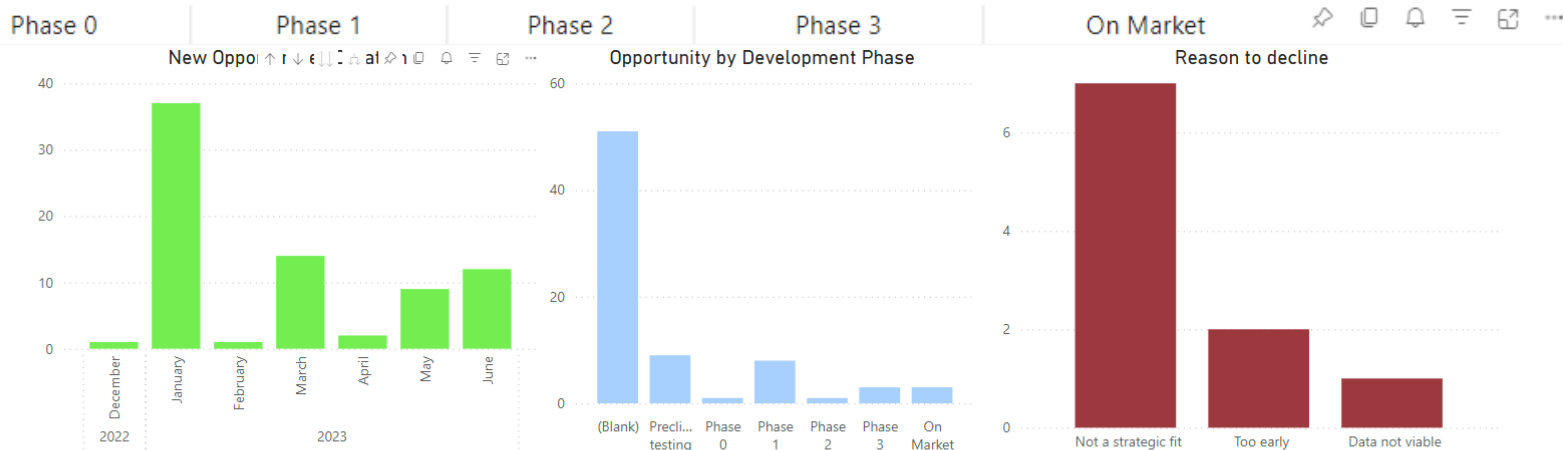
Opportunities by Initiative



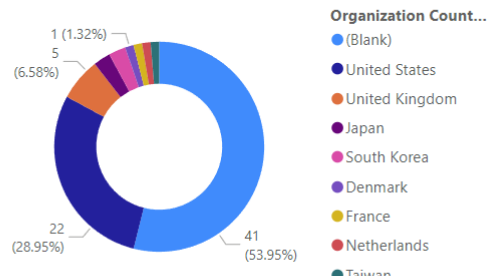
Asset by dev phase and TA



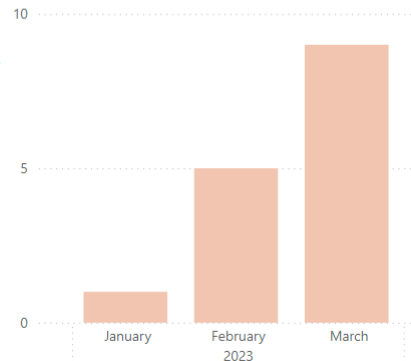
Initiative	Preclinical testing	On Market
Alzheimer	10	0
Cell & Gene	5	4
COVID-19 Research Collaborations	1	0
Immuno-Oncology	3	0
Infectious Diseases	4	2
Melanoma	4	2
Respiratory Syncytial Virus	2	0
<b>Total</b>	<b>29</b>	<b>8</b>



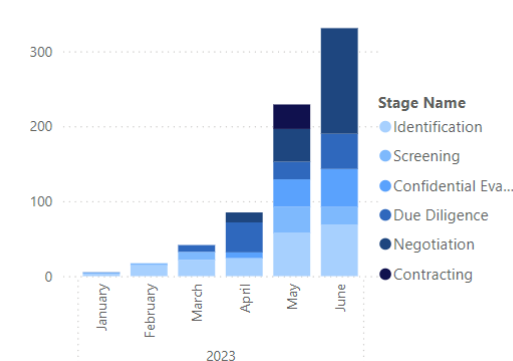
By Country



State Opportunities



Average of Duration by Stage



# Leveraging Advanced Partnering Data



September 24, 2024

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Inpart Summit  
User Group Meeting

Sofia Petta MSc  
Consultant, Citeline | Evaluate



# Introduction and Agenda



**Sofia Petta MSc**

Consultant, Citeline | Evaluate Portfolio Strategy

Advisory Group



## One team, one answer

- Mapping as a service
- Norstella and InPart partnership

## Driving objective decision making in BD&L

- In licensing asset search & screen
- Out licensing partner identification

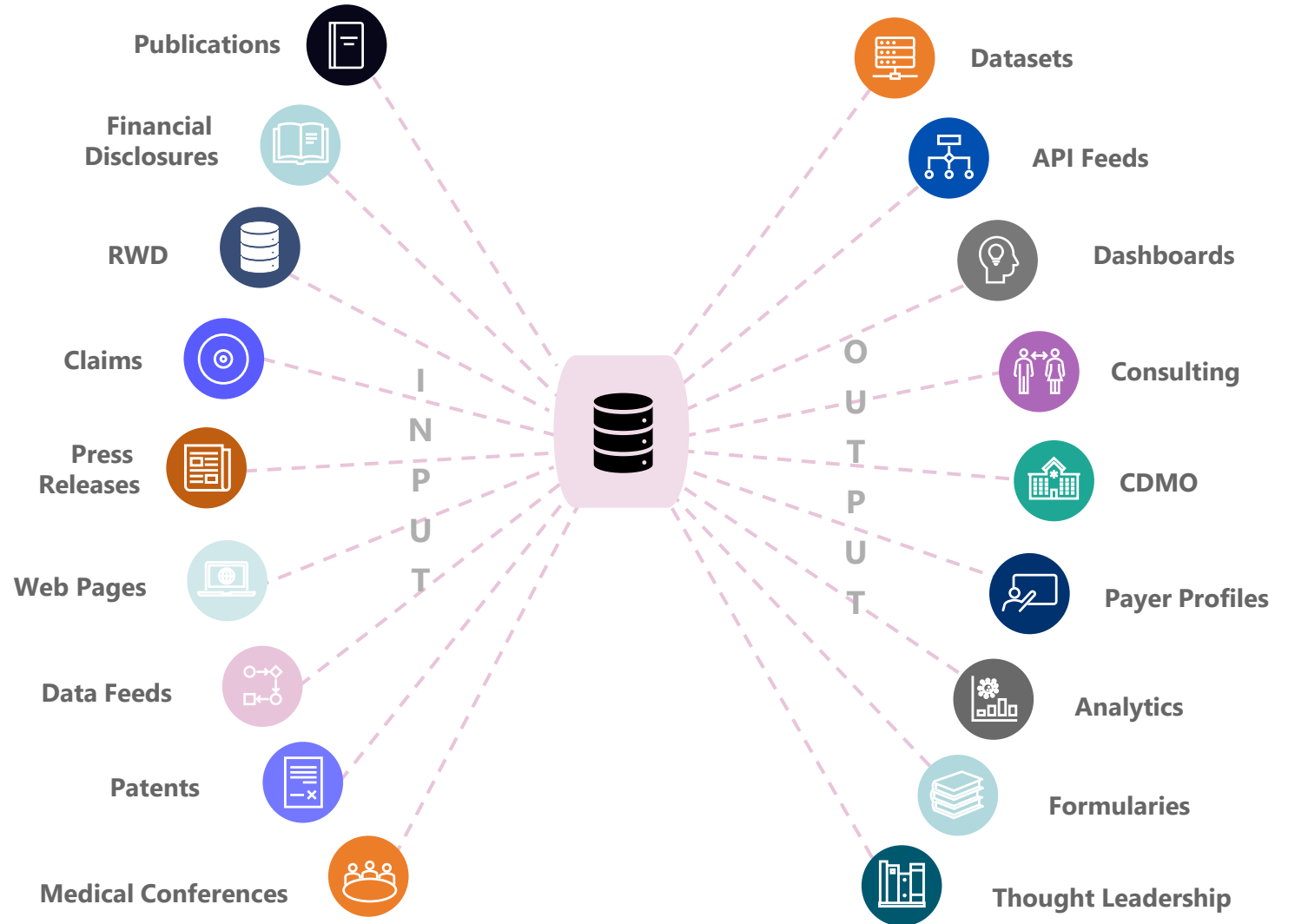
## Data that is:

Market-leading in **scope, timeliness & quality**

Mapped, mastered and **single-sourced**

Available via Snowflake for 3<sup>rd</sup> party integration

## One Team, One Answer



# Leverage data from a trusted partner

## Norstella Mastered Data

**TRIALTROVE**  
CITELINE CLINICAL

Evaluate  
**Pharma**

Evaluate  
**Omnium**

**BIOMEDTRACKER**  
CITELINE COMMERCIAL

**PHARMAPROJECTS**  
CITELINE CLINICAL

- 210k+** Drugs
- 562k+** Trials
- 2.7M+** News Events
- 660k+** Investigators
- 1M+** Drug/regulatory Events
- 640k+** Articles
- 2k+** Formularies
- 8k+** Drug Forecasts

## Delivered via Snowflake



Secure Data Sharing enables the Inpart solutions to seamlessly access the Norstella data universe ensuring the timely delivery of data updates to downstream solutions

## Inpart Integration



Inpart serves Norstella data throughout customer workflows to facilitate data-entry, updates and decision-making



# Evaluate data informs insights on Inpart Extended Asset Profiles

Assessing Opportunity Risk | Product Overview & Profile

## Product | Indication level (Inpart)

**SRP-5051**

Status: Active

Owned by: Echo Z... From: Sarepta T...

Screening: Oct 3, 2023 | Triage: Oct 3, 2023 | Confidential Evalu...: Nov 13, 2023 | Full Assessment

Overview | Asset | Details | Summary | Attachments 0 | Tasks 4 | Meetings 1

### Opportunity overview

**General**

Initiative: Rare disease | Asset type: Pharma asset

Created on: Oct 3, 2023 | Created by: Echo Zhang

**Discovery**

Discovered by: Echo Zhang | Discovered on: Oct 3, 2023

### Overview

Therapeutic category: Musculoskeletal

Therapeutic subcategory: Other musculoskeletal agents

**Indication summary**

Indication	Dev. phase	Status
Duchenne muscular dystrophy	Phase II	Active
Becker muscular dystrophy	Research pro...	Abandoned
Pompe's disease	Research pro...	Abandoned
Progeria/Hutchinson-Gilford sy...	Research pro...	Abandoned

Proprietary level: New molecular entity

Technological category: Biotechnology

Technology: DNA & RNA therapeutics

Mechanism of action: Dystrophin stimulant

## Product overview | Profile (Evaluate)

Product: SRP-5051 : Product Overview | Product Profile

All Financial Data in US \$ (mln)	
Product	SRP-5051
Company	Sarepta Therapeutics
WW Product Profile	SRP-5051 (vesileteplirsen) is an Exon 51 skipping antisense in R&D (Phase II) by Sarepta Therapeutics. In 2030, WW consensus sales are forecast to reach \$175.5 million. Sales of SRP-5051 will account for 0.7% of WW sales for products in EphMRA code M5X (All Other Musculoskeletal Products) in 2030. This product is due to launch WW in Dec 2025. In 2030 it will account for 4.6% of Sarepta Therapeutics's sales. SRP-5051 is currently in R&D (Phase II) (WW Status) for: Duchenne muscular dystrophy [Phase II]; Becker muscular dystrophy [Abandoned - Research project]; Pompe's disease [Abandoned - Research project]; Progeria/Hutchinson-Gilford syndrome [Abandoned - Research project].
Strategy	Organic
Product Status	Active
WW Market Status (Current)	R&D
WW Phase (Current)	Phase II
Generic Name	vesileteplirsen
Other Brand Names	-
Research Codes	PPMO; SRP-5051
Technology	DNA & RNA therapeutics
Pharmacological Class	Exon 51 skipping antisense
Therapeutic Category	Musculoskeletal
Therapeutic Subcategory	Other musculoskeletal agents
EphMRA ATC Code Level 1	M (Musculo-Skeletal System)
EphMRA ATC Code Level 2	M5 (Other Drugs for Disorders of the Musculo-Skeletal System)
EphMRA ATC Code Level 3	M5X (All Other Musculoskeletal Products)
EphMRA ATC Code Level 4	M5X (All Other Musculoskeletal Products)
Biological/Chemical	Biological (BLA)
Therapy Type	Monotherapy
Company's Classification	RNA-targeted therapies PPMO
Originator	Sarepta Therapeutics
Licensee	-
Markets	WW
Routes of Admin.	Injection

# Assessing Development Risk and Combining metrics

Identify risk and evaluate potential companies or assets in a company portfolio with a single view of the Company profile overview page

## Inpart extended profile

**Sarepta Therapeutics**

Type: Pharma, Biotech, ... | Agreements: GDA

Opportunities 2 | Contacts 2 | Meetings 3 | Attachments

**Opportunities 2**

Title	Type / Stage
SRP-6004	To be defined Screening
SRP-5051	In-Licensing Full Assessment

**Sarepta Therapeutics**

Up-to-date • From Evaluate

- Listed
- United States
- Massachusetts • Cambridge
- Dec 31, 1980

R&D projects 40

By: Development phase

Development phase	Count
Phase III	2
Phase II	4
Phase I	1
Pre-clinical	17
Research p...	16

## Company Profile Overview (Evaluate)

Company

Sarepta Therapeutics: Company Overview | Summary

Company Profile

Company	Sarepta Therapeutics
Active or Inactive Company	Active
Listed or Private Company	Listed
Company Classification	Biotechnology
City	Cambridge
State/ Region	Massachusetts
Country	USA
Profile (cell note)	Profile
Evaluate subscriber coverage	Coverage of Sarepta Therapeutics (Active   Listed) Includes: EvaluatePharma® product portfolio; clinical trials; historic financials; venture financing; consensus forecast model; news; company profile.
Marketed	4
Approved	-
R&D Project Count by Phase	Phase III: 2, Phase II: 4, Phase I: 1, Pre-clinical: 17, Research project: 16
R&D Project Count by Therapy Area	Cardiovascular: 1, Central Nervous System: 10, Immunomodulators: 1, Musculoskeletal: 27, Various: 1
R&D Project Count by Technology	Biotechnology: 40
Established Date	12/31/1980
IPO Date	6/3/1997
Ticker	SRPT
Exchange	NASDAQ
ISIN	US8036071004
Former Names	AntiVirals; AVI BioPharma
Acquirer Company	
Company Website	Website

# Discovering the next blockbuster

Whether there is a need to support near term revenue generation in a major market or ensure early access to lock in on an innovative product, Norstellia offer data driven asset search based on market leading commercial and clinical databases

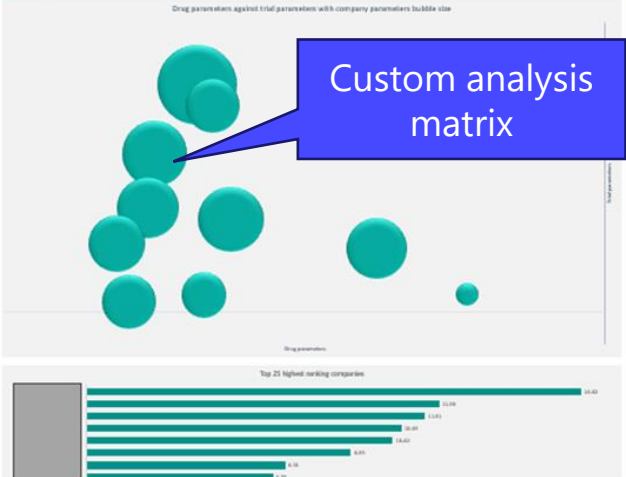
Dynamic weighted prioritization

Asset lists with objective, comparative scoring

Profiling and recommendation



Custom analysis matrix



**Product landscapes**

Therapeutic area landscapes

Number of products by therapeutic area across all drug statuses

Number of products by therapeutic area across all active products

Number of products by cell type within oncology across all drug statuses

Number of products by cell type within oncology across all active products

Directional arrows point to the graphs and tables affected by the dropdown selection

Alternate colors are used to avoid confusion with selectors that are next to each other

Landscaping and contextual analysis

Table 1. CAGR for therapeutic areas

Therapeutic Area	1995-1999	2000-2004	2005-2009	2010-2014	2015-2019	2020-2022
Infectious Disease	15%	17%	23%	17%	11%	11%
Musculoskeletal	8%	7%	14%	7%	7%	7%
Sensory	17%	0%	-4%	2%	14%	10%
Autoimmune	-16%	-7%	6%	-2%	11%	11%
Oncology	2%	3%	3%	3%	3%	3%
Cardiovascular	-2%	-7%	8%	-3%	-1%	-1%
Neurological	2%	3%	3%	3%	3%	3%
Immunology	1%	1%	1%	1%	1%	1%
Endocrinology	1%	1%	1%	1%	1%	1%
Respiratory	1%	1%	1%	1%	1%	1%
Other	1%	1%	1%	1%	1%	1%

Notes: CAGR for total number of products by year bands. Each 10 year period is calculated as the first 5 years against the number of products from 1990-1994 against the number of products in 1995-1999. The only exception to this is the 2020-2022 calculated against the number of products in 2020 and the number of products in 2022.

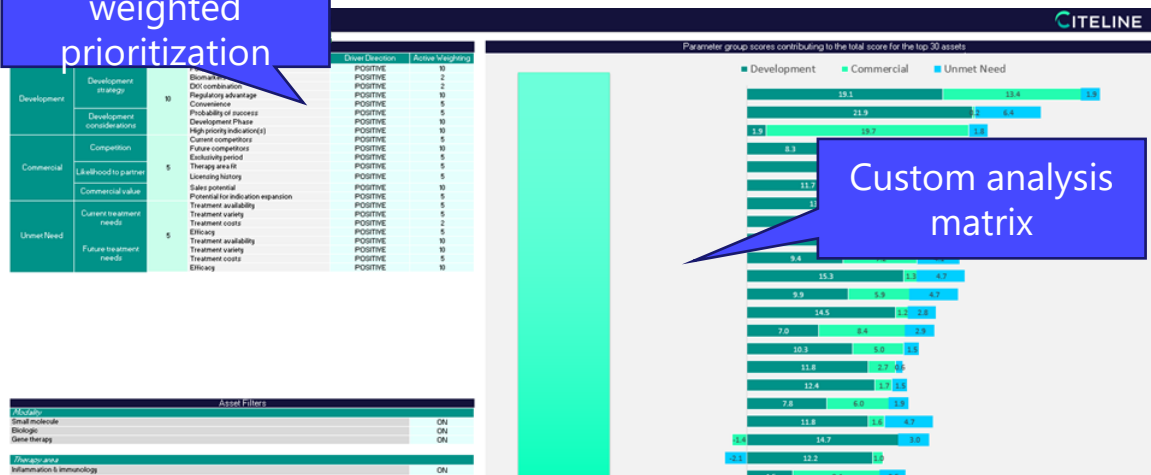
Notes: Infectious disease, musculoskeletal, sensory, autoimmune, and oncology are all areas that have shown growth in the number of products between 2020-2022 ranging from 2% annual growth for oncology to 27% annual increases for infectious disease, likely driven by the COVID-19 pandemic. However, during 2010-2019, cardiovascular and neurological therapeutic areas grew the most (8% and 9%, respectively) aside from miscellaneous and neurology, but have been in decline across 2020-2022 (-11% and -5%, respectively).

Most products are centered around stem cell or t-cell technology, while some more niche cells such as connective tissue cells are also within the autoimmune/inflammation therapeutic area.

# Out licensing partner identification

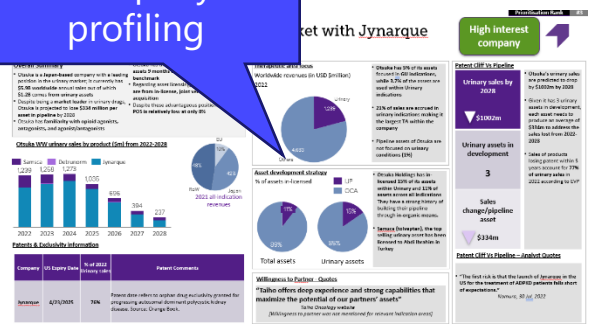
The full suite of Norstell databases contribute to the selection and dynamic prioritization of potential licensee companies. Models are created to be open to changing priorities and scenarios for out-licensing

Dynamic weighted prioritization



Pitch deck optimization

Company profiling

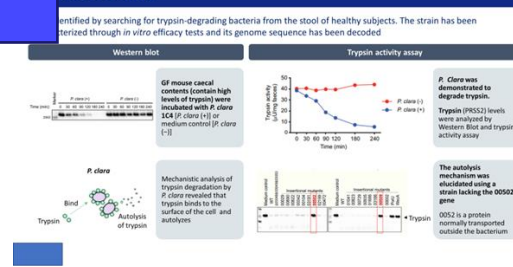


Objective analysis

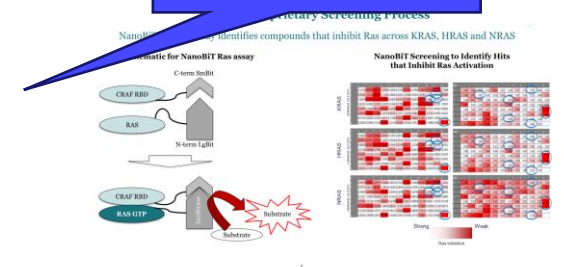
Top 25 partner identification opportunities - Relative

Company	Willingness to Partner	Familiarity with Asset	Time to Market	Probability of Success	Relative Interest
1. AstraZeneca	✓	✓	✓	✓	High interest
2. Amgen	✓	✓	✓	✓	High interest
3. Novartis	✓	✓	✓	✓	High interest
4. Pfizer	✓	✓	✓	✓	High interest
5. Bayer	✓	✓	✓	✓	High interest
6. GSK	✓	✓	✓	✓	Medium interest
7. AbbVie	✓	✓	✓	✓	Medium interest
8. Janssen	✓	✓	✓	✓	Medium interest
9. Sanofi	✓	✓	✓	✓	Medium interest
10. Roche	✓	✓	✓	✓	Medium interest
11. Janssen	✓	✓	✓	✓	Medium interest
12. Takeda	✓	✓	✓	✓	Medium interest
13. Janssen	✓	✓	✓	✓	Medium interest
14. BiMS	✓	✓	✓	✓	Medium interest
15. Viatris	✓	✓	✓	✓	Medium interest
16. Horizon Therapeutics	✓	✓	✓	✓	Medium interest

clinical Findings



Outreach and feedback



## Takeaways and note on H2

*It is more important than ever for dealmakers to have a roadmap for inorganic growth. Be prepared is our message*

- Moving beyond these macro level trends, Norstella provides end to end BD&L support driven by mapped, mastered and singled sourced data
- These data feed quantitative asset and company level metrics **enable** rapid identification, accurate evaluation of target assets, potential licensing partners and much more
- Citeline | Evaluate consultants partner with clients to derive insights from the data and combine outcomes with real world experience to facilitate truly objective decision making



For questions,  
please contact:



Sofia Petta MSc

Consultant, Citeline | Evaluate  
Portfolio Strategy Advisory Group

[Sofia.Petta@norstella.com](mailto:Sofia.Petta@norstella.com)

