Case Study

**CITELINE CONSULTING & ANALYTICS**

# Rare disease prioritization model and landscape analysis

## Situation

A global pharmaceutical company required an objective assessment of multiple rare diseases to identify the most promising indications in which to develop preclinical candidate molecules.

## Approach

Leveraging Citeline's proprietary datasets, our Consulting & Analytics team created a weighted prioritization model to assess each rare disease across multiple areas — including unmet need, market competition, development feasibility, and the client's target product profile — to produce the final ranking and recommendation. Further insight into the highest-ranked diseases was provided by a series of complementary disease landscapes.

# Solution
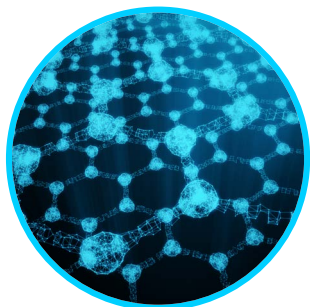
### Prioritization Model
Citeline created a weighted prioritization model to rank indications as the most attractive development opportunities for a set of preclinical assets.

### Ranking Model
The client received an interactive Excel model, ranking multiple rare diseases as opportunities in which to pursue the development of its portfolio of preclinical candidates.

### Parameters
Weightings could be set by the user for four broad parameter groups and over 30 specific sub-parameters.
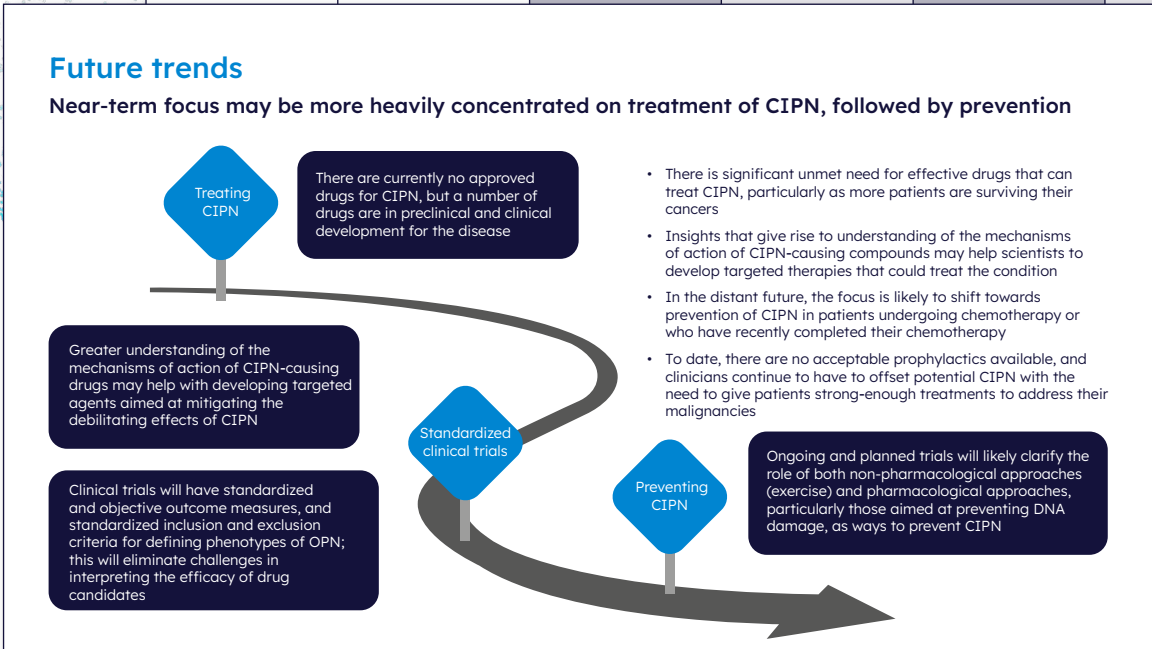
### Model Outputs
Model outputs were illustrated using dynamic bubble plots and heat mapping to facilitate the interpretation of final scores.

# Impact

We developed a rigorous prioritization model and complemented it with in-depth disease analysis. The ranking not only highlighted the most promising indications for preclinical candidate molecules but also provided actionable recommendations to guide the client's strategic decision-making. Armed with insightful intelligence, the client could confidently allocate resources and prioritize initiatives that aligned with its overarching goals and objectives.

# Example Deliverables

Unmet need and market factors against development feasibility

Development feasibility

B
A
Most attractive indications
D
C
J
G
I
E
A
A

Least attractive in...

| Ranked Indications | | Categorized scores | | | |
|---|---|---|---|---|---|
| Rank | Indication | Final category | Unmet need | Market factors | Development feasibility |
| 1 | C | A | C | C | B |
| 2 | I | A | C | A | B |
| 3 | J | B | B | B | B |
| 4 | G | B | C | C | B |
| 5 | F | C | A | A | E |
| 6 | D | C | C | E | B |
| 7 | H | C | B | D | E |
| 8 | B | E | E | B | B |
| 9 | A | E | D | E | D |
| | | | | | E |

## Future trends

**Near-term focus may be more heavily concentrated on treatment of CIPN, followed by prevention**

Treating CIPN

There are currently no approved drugs for CIPN, but a number of drugs are in preclinical and clinical development for the disease

Greater understanding of the mechanisms of action of CIPN-causing drugs may help with developing targeted agents aimed at mitigating the debilitating effects of CIPN

Standardized clinical trials

Clinical trials will have standardized and objective outcome measures, and standardized inclusion and exclusion criteria for defining phenotypes of OPN; this will eliminate challenges in interpreting the efficacy of drug candidates

Preventing CIPN

Ongoing and planned trials will likely clarify the role of both non-pharmacological approaches (exercise) and pharmacological approaches, particularly those aimed at preventing DNA damage, as ways to prevent CIPN

- There is significant unmet need for effective drugs that can treat CIPN, particularly as more patients are surviving their cancers
- Insights that give rise to understanding of the mechanisms of action of CIPN-causing compounds may help scientists to develop targeted therapies that could treat the condition
- In the distant future, the focus is likely to shift towards prevention of CIPN in patients undergoing chemotherapy or who have recently completed their chemotherapy
- To date, there are no acceptable prophylactics available, and clinicians continue to have to offset potential CIPN with the need to give patients strong-enough treatments to address their malignancies

# Value

**Subject-matter expertise** on a range of therapy areas to partner with you on your bespoke business needs
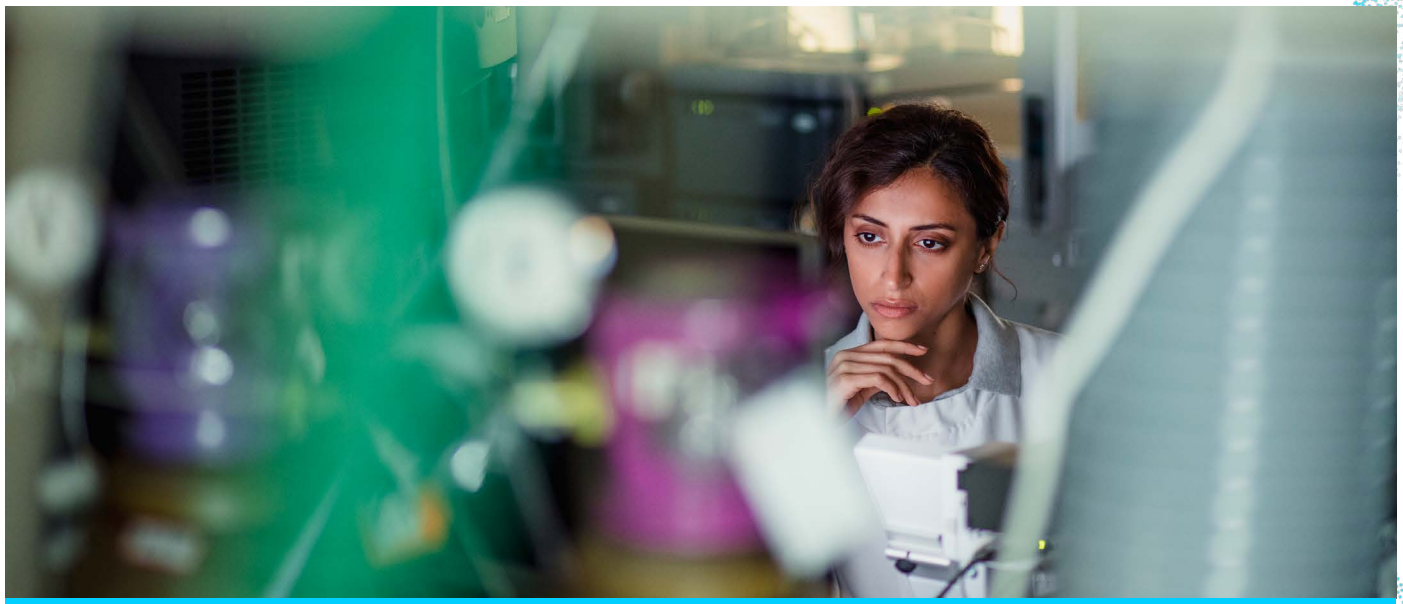
**Leveraging market-leading** products and real-world data (RWD) solutions

**Validating, benchmarking, and refining** data to assess feasibility

**Accurate and credible** recommendations you can trust for successful outcomes

Leverage the Citeline Consulting & Analytics team to assess the rare diseases landscape, using our deep domain expertise and access to extensive databases to compile a holistic view of the current landscape with state-of-the-art visualizations and insights.

**LEARN MORE**

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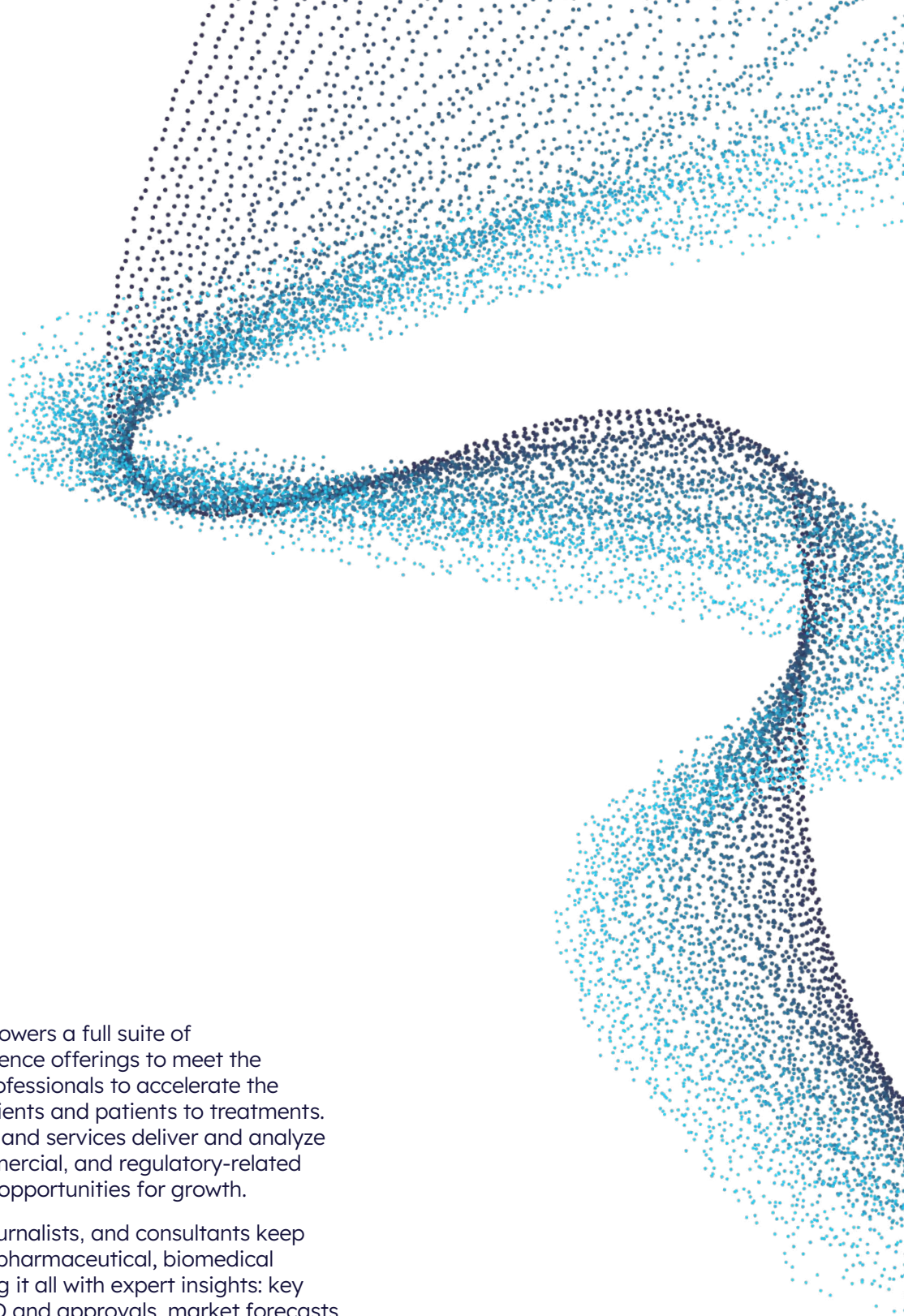
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