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Where data means more

CASE STUDY

Data-Driven Solutions for Rare Monogenic Blood Disorders: Identifying and Prioritizing Treatment Compounds for Improved Patient Outcomes

Client's Challenge and Goal

The client, was working on identifying potentially effective drugs for the treatment of rare monogenic blood disorders. These disorders are caused by single gene mutations, making the search for suitable treatments complex. Recognizing the need for specialized expertise, the client sought out Excelra for support.

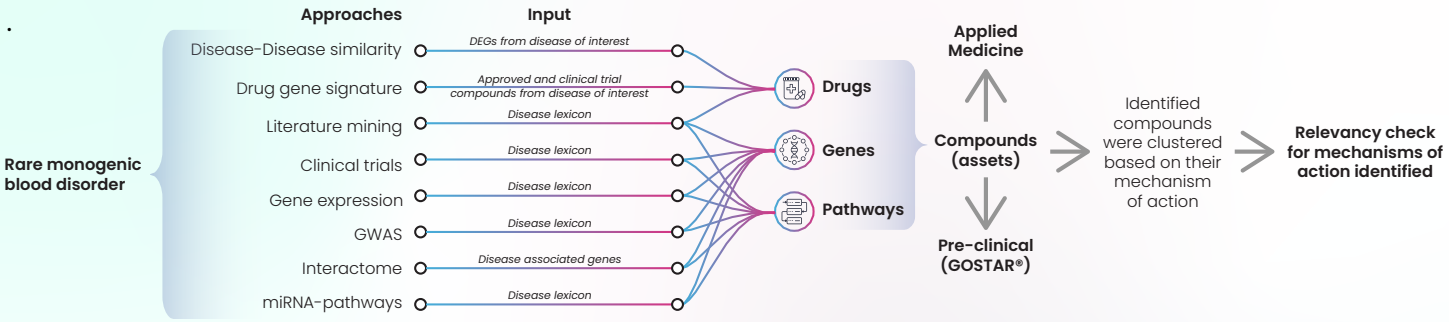
Our Client

An established Pharma company based out of US.

Our Approach

To address these challenges, Excelra embarked on a comprehensive research journey. We delved into existing literature and datasets, meticulously examined disease-drug correlations. Employed a range of methodologies, Excelra explored disease similarities, drug-gene signatures, and genome-wide association studies (GWAS) to uncover valuable insights.

Excelra had to extract pertinent information from diverse sources. This wealth of knowledge was then utilized to create disease-drug pairs based on their mechanism of action. By thoroughly understanding the underlying genetic mutations and the corresponding drug mechanisms, Excelra aimed to provide the client with a targeted and informed approach towards identifying potentially effective drugs for rare monogenic blood disorders.

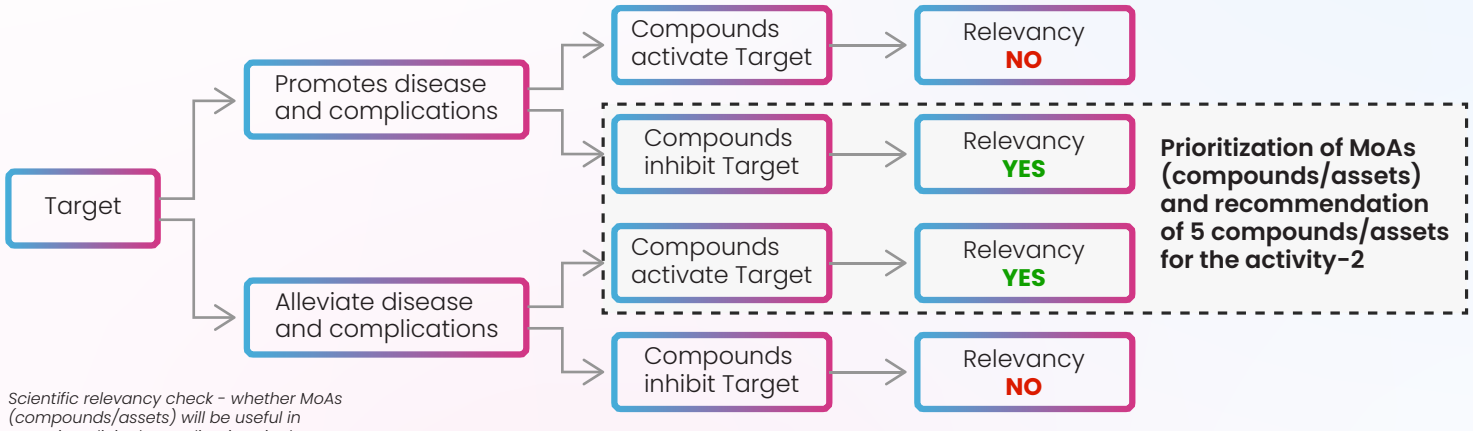


Approaches for asset identification in relation to rare monogenic blood disorders

The top five mechanisms of action were selected, and drugs were prioritized for each of them. An in-depth analysis of each of the MoAs was completed considering the following points:

- Relevance of targets and MoAs in the disease
- Clinical or preclinical scientific evidence
- Known literature on animal models, target safety, and hypotheses availability

Following this stage, the best drugs for each mechanism of action were recommended to the client, and the MoAs and compounds were prioritized according to Excelra's prioritization process. Excelra conducted relevancy check on the mechanisms of action for compounds/assets to discover if they promoted or alleviated disease and complications.



Prioritization of MoAs and compounds/assets

Our Solution

Excelra played a crucial role in identifying effective drugs for the treatment of rare monogenic blood disorders. We utilized various approaches which included analyzing disease similarities, drug-gene signatures, and conducting genome-wide association studies (GWAS). This approach established correlations between diseases and potential drugs.

The top five mechanisms of action (MoAs) were selected, and drugs were prioritized for each of them. Excelra conducted a thorough analysis of each MoA, considering factors such as the relevance of targets and MoAs in the disease, existing scientific evidence, and literature on animal models and target safety.

Based on the analysis, Excelra recommended the best drugs for each MoA to the client. They also conducted a relevancy check to ensure that the identified compounds/assets had a positive impact on the disease and its complications.

The results of Excelra's approach were impactful. The client received targeted recommendations for potential disease-drug pairs based on their mechanism of action. The prioritization process ensured efficient resource allocation, and the relevancy check added confidence to the proposed compounds/assets.

Conclusion

Excelra's support and methodology proved valuable in addressing the challenges of finding effective drugs for rare monogenic blood disorders. Our expertise in data analysis and prioritization make us a valuable partner for pharmaceutical companies seeking similar solutions.