

Attaining Insights on the Rare Disease Drug Development Landscape

Drug development is a complex endeavor, particularly within rare diseases. Given the additional challenges impacting rare disease R&D, the ability to easily generate critical insights and hone your development strategy is key. Through Pharmaprojects, effectively assess the rare disease landscape, including identification of:

Key Players With Orphan Drug Status

Drugs with Orphan Drug Status are easily identifiable in Pharmaprojects by using the **Event Type** field.

Through the Orphan Drug Designation Program, various development incentives are available to qualifying drugs targeting rare diseases, including exclusivity rights and cost benefits, protocol assistance from FDA, and potential decreased wait-time for drug approval.

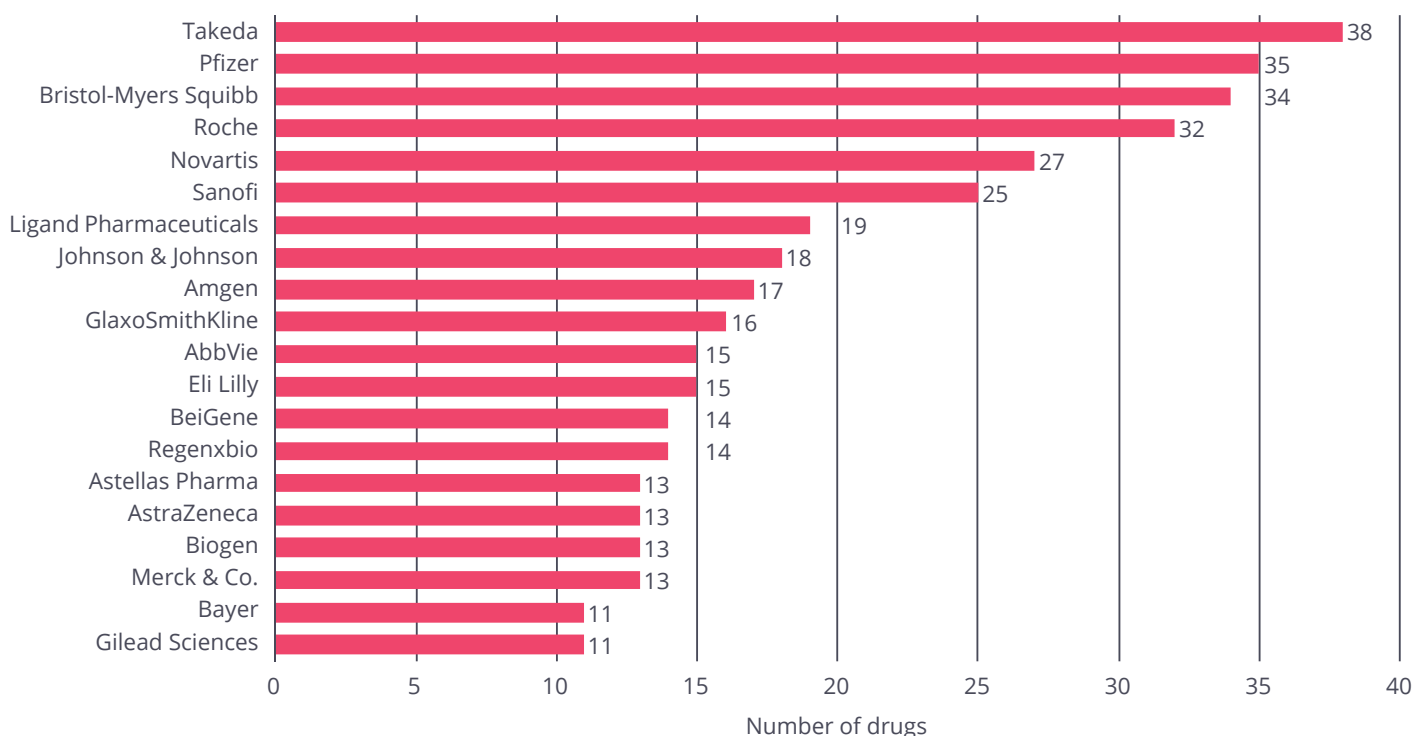
A client interested in identifying Orphan Drug companies reached out to our analyst team via **Ask The Analyst**, which is included within Pharmaprojects and provides direct access to our

industry experts. The analyst provided an overall strategy as well as suggestions to further refine the search.

Searching for drugs in **Pipeline** development (Global Statuses spanning Preclinical to Pre-registration) that have been granted **Orphan Drug Status** provides insights into these players possessing a potential advantage within the challenging rare disease space.

Overall, **1,292 companies** are developing **1,387 drugs** that have received Orphan Drug Status.

Top 20 Companies Developing Orphan Drugs



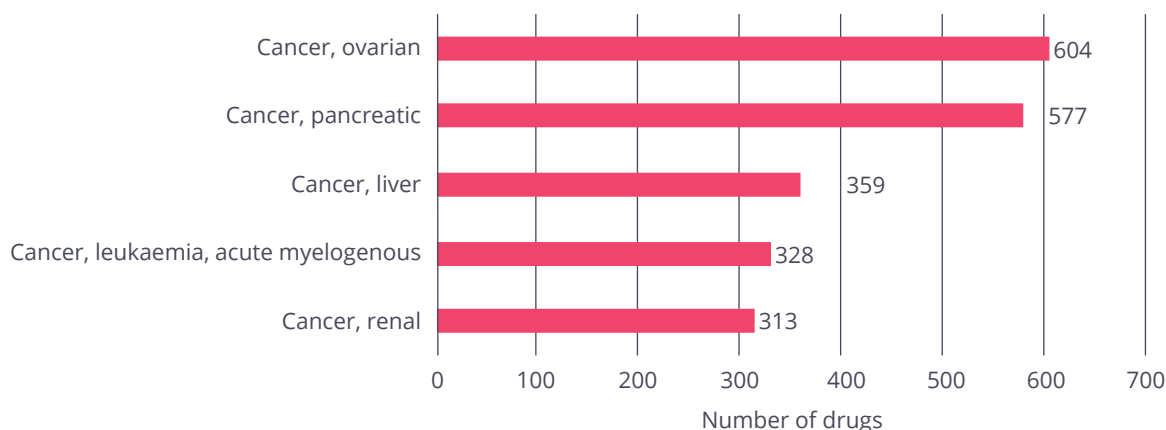
“Fallen Angel” Drugs for Rare Diseases

Quickly search for drugs targeting rare diseases using the **Drug Disease Group** field

Less than 10% of potential new drugs actually make it to market, resulting in a vast pool of “fallen angel” drugs that have already undergone early stages of research and potential candidates for repurposing.

Identify “fallen angel” drugs for rare diseases by searching for Global Statuses of **Discontinued** or **No Development** Reported in the **Rare Diseases** Drug Disease Group – a total of **7,397** drugs.

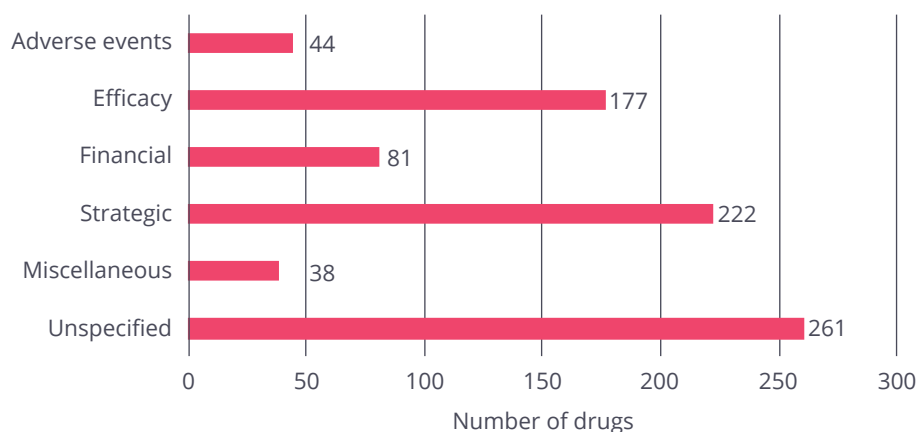
Top 5 Diseases with “Fallen Angels”



The search can be refined, such as limiting by

- Therapeutic group to focus on anticancer drugs, reducing the pool of candidates to **2,629** drugs
- Highest development status reached by the drug before discontinuation, such as Phase III, which returns **241** drugs
- When the drug dropped out of development to identify recent activity, including discontinuations between 2015-2017, which includes **1,652** drugs

Further inform your research by reviewing reasons behind discontinuation. When publicly disclosed, Pharmaprojects captures these details within the Drug Event Details for the Event Type **Discontinuation Confirmed**, including:



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