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Accelerating Rare Disease Drug Repurposing.

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Case Study: Accelerating Rare Disease Drug Repurposing

Type of Organization: Biotech Startup

Industry: Pharmaceutical / Rare Diseases

The Challenge: Identifying existing, approved drugs that could be repurposed for a very rare, previously untreatable orphan disease. Traditional methods involved labor-intensive manual literature reviews and speculative *in vitro* screening, which were slow and often unproductive due to limited mechanistic understanding of the disease.

The ScieFI Solution: The startup deployed **ScieFI 's Adaptive Knowledge Graph (AKG)** and **AI Co-Scientist Module (ACS)**. The AKG ingested all available public omics data, patient registry data, and drug databases, including off-target interaction profiles. Its **cross-disciplinary semantic bridging** identified subtle molecular commonalities between the rare disease's pathology and known mechanisms of action for existing drugs in unrelated therapeutic areas. The ACS's **Intelligent Hypothesis Generation** then proposed a prioritized list of repurposing candidates with high confidence scores, complete with predicted molecular interactions. It further designed a streamlined set of *in vitro* validation experiments, outlining optimal cell lines and readouts.

Impact & Benefits: This approach drastically reduced the initial drug screening phase from over a year to just three months. The startup identified two highly promising drug candidates for rapid preclinical validation, one of which had already completed Phase II trials for a different indication, significantly de-risking and accelerating the path to patient impact for a previously neglected disease.

Key Features Highlighted:

- o Adaptive Knowledge Graph (AKG)
- o Cross-disciplinary semantic bridging
- o AI Co-Scientist Module (ACS)
- o Intelligent Hypothesis Generation
- o Automated Experimental Design & Optimization