



KECK GRADUATE INSTITUTE
of Applied Life Sciences



The Center for Rare Disease Therapies

Keck Graduate Institute *of Applied Life Sciences*

Keck Graduate Institute (KGI) is the only graduate institution in the U.S. created exclusively to educate scientists and engineers to be leaders in the bioscience industry.



KGI.edu

Why KGI as a Site for the Center?

KGI is a unique organization established to deliver programs differently from traditional research institutions. We are setting the standard for team-based, hands-on, real world education at the intersection of science and business.

Mission: KGI is dedicated to education and research aimed at translating into practice, *for the benefit of society*, the power and potential of the life sciences.

Faculty: most faculty come to KGI with industry experience and focus on applied development

Students: our students prepare to become new leaders of the biotech and biomedical industry, including a required industry internship..

Curriculum: Every class focuses on industry challenges, from management to drug discovery, from bioprocessing to bioethics.

Industry Relationships: KGI has extensive industry relationships through its Board of Trustees, Advisory Council, sponsored research and team master's projects.

Team Master's Projects (TMPs): Industry-sponsored real world projects are required as part of the MBS (Master of Bioscience) degree program

The KGI Capstone: Team Masters Project (TMP)



Team Masters Project: Amgen



Team Masters Project: Beckman Coulter

Real-world projects with life science companies

62 projects to date

Participating companies include:

- **Beckman Coulter (7)**
- **Amylin (4)**
- **Amgen (3)**
- **Gilead Sciences (5)**
- **Ortho-Clinical Diagnostics (2)**

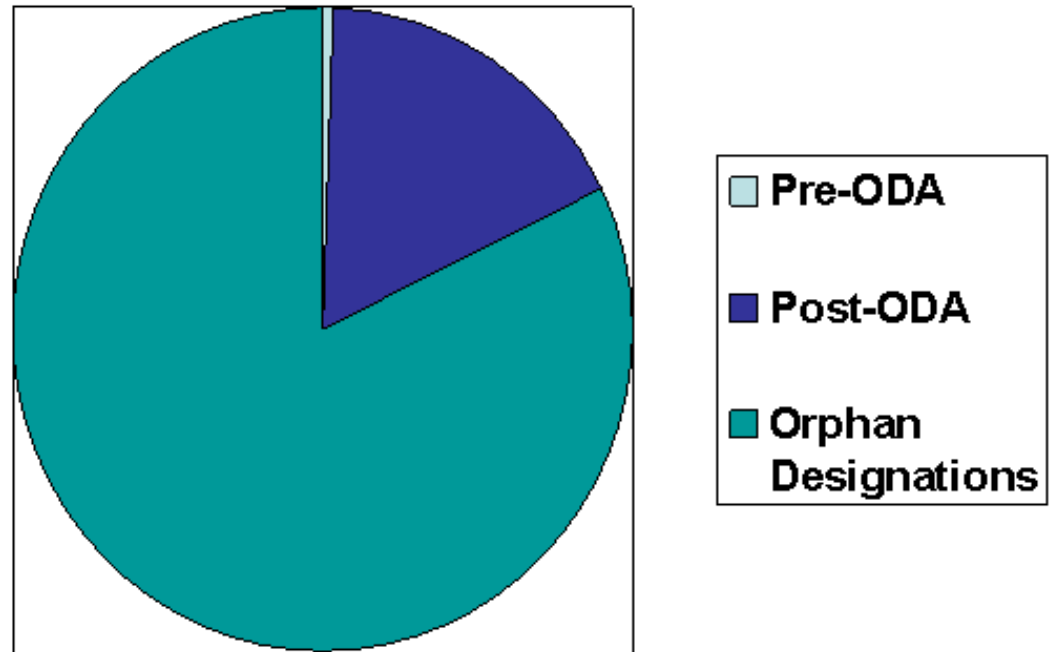
The Challenge: *much more to be done*

- Over 7000 known rare diseases

Orphan Drug Designations

Since 1983:

- Over 2400 orphan drug designation requests
- 1700 orphan drug designations
- Over 300 approved orphan products





The Center for Rare Disease Therapies

MISSION

The mission of the Center for Rare Disease Therapies is to promote the development and commercialization of treatments for rare diseases.

By matching promising technologies with corporate and philanthropic partners, the Center will act as a catalyst for reviving potential therapies and fostering new ones.

Our Goal: more therapies for rare diseases



Functions of the Center:

What will we do?

- **Profile Rare Diseases** - information on the basic science of the disease, research programs to date, published clinical data, and prevalence studies.
- **Identify promising opportunities** – investigate orphan drug filings, outreach to patient advocates, engage key companies.
- **Assess drug leads and prioritize** – aim is to pursue leads with the greatest chance of making a difference: *severity of disease – potential of treatment – access to asset-holder.*
- **Facilitate licensing, development, and/or partnership options** through non-profit alliances with corporate and philanthropic partners.
- **Sponsored projects** – reducing opportunity-cost by helping companies address issues at key stages of drug development.

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