Helping to combat rare diseases suffered by approximately 25 million people in the United States has become a top priority at Keck Graduate Institute.

Having held its inaugural advisory board meeting back in October, KGI's new Center for Rare Disease Therapies is now going forward looking to help bring solutions to an unmet demographic of rare disease patients.

“Our goal is to raise awareness about rare diseases and to bring the skills of KGI team education approaches to the problems of finding new therapies,” said Ian Phillips, the center’s director and Norris Professor of Applied Life Sciences.

KGI President Sheldon Schuster shares Mr. Phillips’ sentiments and looks forward to creating and strengthening relationships with other organizations.

“We are thrilled about the opportunity to work with the National Organization for Rare Disorders, patient groups, the bioscience industry, government agencies and other academic partners to build this program focused on rare diseases,” President Schuster said.

“We are excited about the education and research opportunities that this center will provide and are hopeful that we can catalyze the development of new therapies.”

Approximately 7000 rare diseases are accounted for in the United States today according to the U.S. Food and Drug Administration. A disease is considered rare if fewer than 200,000 people have it.

Before the Orphan Drug Act of 1983 was passed, only 10 treatments had been developed for rare diseases in the decade prior. Within the past 25 years, more than 300 treatments have been FDA approved.

Yet Mr. Phillips feels a significant number of FDA-approved drugs that could possibly benefit patients are not as accessible as they should be. One of the objectives of the new center will be to help find ways to help make remedies more obtainable.

“More than 1800 drugs that could help those diagnosed with a rare disease have received orphan drug designation from the FDA, but for various reasons only 326 of these therapies are available to patients,” Mr. Phillips explained. “The Center for Rare Diseases at KGI is devoted to developing ways for more therapies to become available to those who need them.”

Biotech industry notables Gregg LaPointe (Sigma-Tau Pharmaceuticals CEO), Timothy Cote (FDA Office of Orphan Product Development director), Diane Dorman (National Organization for Rare Disease Center for Rare Disease continues on the next page)

Disorders vice president for public policy), Dan Maher (BioMarin Pharmaceutical vice president of product development) and Dennis Fenton (KGI Board of Trustees member and former Amgen executive) were speakers at the October board meeting. The center’s board of advisors also discussed the needs of rare disease patients and how to incorporate KGI faculty and students into the mix as part of the solution.

Mr. Fenton pointed out a priority of the KGI Center is to enhance student understanding of rare diseases, which will in turn help expand opportunities for the creation of new remedies after KGI students graduate and move further within the bioscience industry.

“As we set things up, we are making sure that what we're doing fits in with the mission of KGI, especially the teaching environment,” he said. “We had to be sure that this is primarily about educating students.”

Apart from education, KGI will also look to act as a facilitator and research consultant of rare disease therapies. With the majority of created drugs never moved from the laboratory to store shelves and the number of rare disorders continues to increase, the endeavor will be long term.

“Such an effort requires working with patient groups, companies and government agencies,” Mr. Phillips said.

In its beginning stages, the Center for Rare Disease Therapies will not focus on specific diseases, but cover a broad range of disorders in its research. The center will also continue to collaborate with industry experts in both the public and private sectors.

“Our next step is to see where we will focus our energy,” Mr. Fenton said. “You can’t address all diseases so you have to narrow it down. It’s definitely fun to be a part of this.”

—Landus Rigsby