

Former FDA Official Tim Coté Joins KGI Faculty

A former high-level official with the U.S. Food and Drug Administration (FDA) has joined Keck Graduate Institute's (KGI) faculty, bringing extensive knowledge and experience in clinical regulatory affairs.

Dr. Tim Coté, former director of the FDA's Office of Orphan Products Development, will split his time between teaching at KGI and serving as chief medical officer for the National Organization for Rare Disorders (NORD).

"I was attracted to KGI because it is one of the only academic institutions active in the orphan drug sphere," said Coté, who recently retired after 22 years with the federal government, including eight years at the FDA.

Coté is no stranger to KGI, having worked on special workshops with the Center for Rare Disease Therapies, which is dedicated to promoting the development of orphan drug therapies that target rare diseases affecting nearly 30 million Americans or approximately 1 out of 10 people.

"This is really a big advance for KGI in terms of capability and visibility, adding someone like Tim who has such extensive and recent experience at the FDA," said KGI President Sheldon M. Schuster, PhD, who also serves on the NORD Board of Directors. "His appointment brings not only awareness from outside KGI but also brings a level of experience that's going to be available for our students that nothing else can match."

(continued on back page)



*Professors
Ian Phillips
and Tim Coté*

CRDT Welcomes New Co-Director

This year we are very fortunate to have Tim Coté, MD, MPH, the former director of the Office of Orphan Products Development at the Food and Drug Administration, join the KGI faculty and accept the co-directorship of the Center for Rare Disease Therapies (CRDT). His knowledge and experience, plus his joint appointment as medical director of the National Organization for Rare Diseases (NORD), will be of great benefit to the Center and the rare disease community it supports.

By working with the government, industry and patient advocates, the CRDT seeks to increase the number of therapies available to patients, while raising awareness of rare diseases and promoting new treatments through the Orphan Drug Act.

Major achievements of the Center have included efforts to increase orphan drug designations through practical courses at KGI and hosting FDA workshops on the drafting of Orphan Drug applications by companies and patient advocate groups.

For more information about the Center, please call (909) 607-7692. Or visit our website: www.kgi.edu/crdt

Ian Phillips, PhD, DSc, FAHA
Norris Professor of Applied Life Sciences and Co-Director,
Center for Rare Disease Therapies



KGI to Host 3RD Annual FDA Workshop

Tentatively scheduled for Feb. 28-29

Worried about creating an orphan drug designation application?

You're not alone. Typically, submissions to the FDA evoke considerable consternation among drug sponsors. This workshop is designed to make the process of orphan drug development transparent and accessible to those uninitiated in regulatory affairs.

The chief task of the orphan status designation application is to convince the FDA's Office of Orphan Products Development (OOPD) of two things: that the proposed drug is for a rare disease (i.e. that fewer than 200,000 persons in the U.S. have the rare disease or condition) and that there is a medical rationale for believing the proposed drug has "promise" for treating the rare disease/condition (i.e. clinical data, animal model data OR, rarely, in vitro data, but not exclusively theoretical considerations). Orphan status designation can be secured any time prior to the submission of a marketing application; it need not be under consideration as an investigational new drug (IND) nor are preclinical toxicology studies nor manufacturing certifications required.

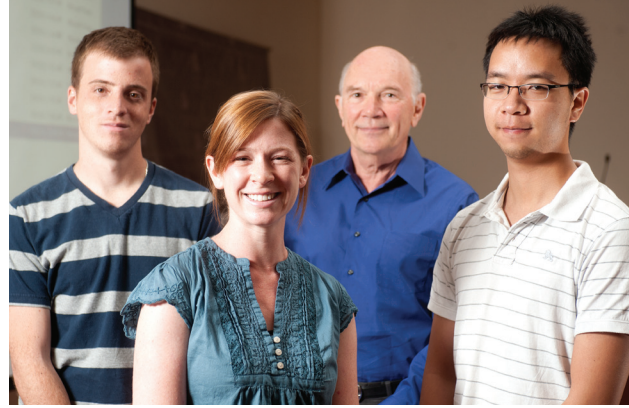
If you have a product envisioned for treatment of a rare disease that you want to see move forward and there is sufficient data to show it may be promising, then you're ready for this workshop.

At the 2012 workshop (tentatively scheduled for Feb. 28-29), officials from OOPD and other FDA staff will directly engage participants in the practical matter of constructing an application for orphan status designation. Finalized applications will be submitted to the FDA on site at the close of the workshop. Attendees should note that participation in the workshop confers neither expedited processing nor preferential consideration of their applications.

All applications are confidential, and during the workshop participants will have private rooms to work in. There is guaranteed time for each participating group or company to meet face to face with FDA officials.

*Daniel Burke, Laura Wilson,
Professor Ian Phillips and Rudy Tsay*

Students Aid Search for Lymphomatic Malformation Cure



Three KGI premed students turned a classroom assignment into a successful effort that could eventually help save the lives of hundreds of small children suffering from lymphomatic malformation.

Daniel Burke, Laura Wilson and Rudy Tsay wrote an Orphan Drug Designation Application on behalf of researchers at the University of Iowa hospitals and clinics for OK-432, a substance that has proven to be effective in treating lymphomatic malformation, a rare and fatal disease that causes large growths on the heads and necks of young children who eventually suffocate from constricted airways.

The students wrote the application for the course “Writing an Orphan Drug Designation,” taught by Ian Phillips, PhD, KGI’s Norris Professor of Applied Life Sciences.

Phillips is director of KGI’s Center for Rare Disease Therapies, which is dedicated to educating and advocating for therapies for rare diseases, which afflict nearly 30 million Americans. Of the 7,000 identified rare diseases, many have no treatments. For those that can be treated, the cost to the patient is often prohibitive.

“This fits exactly with the mission of the Center for Rare Disease Therapies,” said Phillips, adding that he was proud of the students’ accomplishment.

To receive orphan designation, the proposed drug therapy must target a rare disease (one with a patient population of 200,000 or less) and it must show promise for treating the disorder. OK-432 is manufactured in Japan and was originally developed to treat certain forms of cancer, with moderate success.

While U.S. Food and Drug Administration (FDA) approval of the application does not guarantee the drug itself will be approved, it allows researchers to seek grant funding to conduct further clinical trials and provides incentives to the existing manufacturer to increase production of the drug or for other companies to develop the treatment.

Burke, whose mother is a research nurse at the University of Iowa, took the lead on the project. She coordinates clinical trials and studies on OK-432 for Dr. Richard Smith, who

has been testing the treatment under an FDA Investigational New Drug application for more than a decade.

OK-432 consists of a series of four injections with a 96% success rate in treating lymphomatic malformations, which is very rare with only about 100 new cases a year.

“This opens up a lot of opportunities for a drug that has been stuck in clinical trials for the past 10 years,” Burke said.

Tsay said his group benefited from an orphan drug designation application workshop conducted by the FDA at KGI last November. They used a checklist provided by the FDA as a template for their application. Some of the rest of it was luck.

“The situation just fit perfectly for us,” Tsay said. “We had a good sponsor drug for a very small subset of the population.”

Wilson was surprised at the number of drugs that might benefit from orphan drug designation if more people were aware of and understood the process, how easy it is to write an application and how satisfying success feels.

“It’s a very disfiguring disease that this drug helps to treat,” Wilson said. “What more can you ask for in terms of having a real world impact.”

Wilson and Tsay landed internships with the FDA and worked with the Office of Orphan Products Development.

All three students came to KGI for the Postbaccalaureate Premedical Certificate (PPC), a one-year program designed to give premed students the competitive edge in getting into the medical school of their choice. And all three have decided to take advantage of the PPC program’s option of staying an additional year to earn a Master of Bioscience (MBS) degree while applying to medical school.

Burke, Tsay and Wilson are not the first KGI students to be involved with successful orphan drug designation applications. Silviya Meletath (MBS ‘11) helped win FDA approval last year for BPT Pharmaceuticals in Irvine for a drug called ProEnzy that shows promise in treating pediatric Multiple Sclerosis.

Workshop Leads to New Orphan Drug Opportunities

Thanks to a workshop co-sponsored by KGI's Center of Rare Disease Therapies, NATCO Pharma Ltd has become the first Indian company to receive approval from the U.S. Food and Drug Administration (FDA) for an Orphan Drug designation application.

NATCO was one of 21 teams from pharmaceutical and biotech companies, as well as from universities that met with FDA representatives earlier this year at KGI to learn step-by-step how to write and file an Orphan Drug designation application.

A team of academics from Oregon Health and Science University in Portland also was among those who submitted Orphan Drug designation applications that received FDA approval.

Dr. Praveen C. Myneni, Coordinator-Drug Development for NATCO, said the company submitted three separate applications for a compound called NRC-AN-019 to treat Chronic Myelogenous Leukemia (CML), pancreatic cancer and Glioma, a form of brain cancer. The

compound fit the FDA's parameters for Orphan Drug designation since it targets a rare disease (one with a patient population of 200,000 or less) and it shows promise for treating the rare disease.

Rare diseases affect nearly 25 million Americans. No treatment is available for most of the 7,000 identified rare diseases, and for those that can be treated, the cost to the patient is often prohibitive.

While Orphan Drug designation does not guarantee drug approval, it allows researchers to seek grant funding to conduct further clinical trials and provides incentives to the existing manufacturer to increase production of the drug or for new manufacturers to develop the drug.

Currently, NATCO is conducting a clinical trial for NRC-AN-019 in India. Based on the results, the company plans to start a clinical trial in the United States later this year.

Myneni, who read about the workshop on the FDA website, said, "It was a unique opportunity to interact with FDA officials and receive their guidance. It was so much more than we expected."

Dr. Ed Neuwelt, a professor of neurology and neurosurgery at Oregon Health and Science University in Portland and his team were the only workshop participants who were strictly academics with no commercial funding nor interest in a company.

“It was a unique opportunity to interact with FDA officials and receive their guidance. It was so much more than we expected.”

They received Orphan Drug designation to use ferumoxytol, an iron oxide nanoparticle, as a contrast agent for magnetic resonance imaging of glioblastoma, a malignant brain tumor.

Neuwelt said ferumoxytol is much more accurate than the “gold standard,” gadolinium-based contrast agent, for measuring the blood volume of brain tumors and in differentiating between an active tumor and pseudo inflammation in response to radiation and chemotherapy, which occurs in 30 to 50 percent of patients.

“Pseudo progression is a good indication that you want to maintain the current therapy,” Neuwelt said. “If it’s real progression, you will want to change the therapy.”

Project Manager Lisa Bennett, who helped write the application, said the team arrived at the workshop with a rough draft of the application and met with FDA officials four times over two days to make revisions.

“Our application never would have been in that good a shape if we had not gone to the workshop,” Bennett said.

Six, federally-funded clinical trials on ferumoxytol are under way. Neuwelt said the next step is a meeting with the FDA to plan for marketing approval.

Ian Phillips, PhD, Norris Professor of Applied Life Sciences and co-director of the Center for Rare Disease Therapies, said the FDA workshops started

with educational workshops for KGI students. Thirty pharmaceutical companies and patient advocates attended the first commercial workshop in February 2010.

The KGI model was repeated later that year at University of Minnesota and in Washington, DC. This year, the FDA has expanded the workshops to overseas locations including India and the United Kingdom. The next FDA workshop at KGI is planned for Feb. 28-29, 2012.

“These workshops are a highly efficient way to get Orphan Drug designation applications done correctly and into the hands of the FDA for approval, which fits perfectly with the center’s mission,” Phillips said.

By Elaine Regus

Big Pharma's New Interest in Rare Diseases

For many years the highly profitable pharmaceutical companies had no reason to pay attention to the rare disease community. The rare disease market by definition was small. It did not offer the profits of drugs like antihypertensive drugs where the market was over 25 million patients. However, nothing stays the same.

The number of drugs in the pipeline from big companies is diminishing. In 1996, there were 57 new drugs approved by the FDA, but by 2010, there were only 19 new ones. Fewer of these drugs were blockbuster drugs.

Then, in 2005, a surprising statistic appeared: the number of orphan drugs approved was equal to the number of non-orphan drugs that had received FDA approval.

Clearly, a new model was emerging. Orphan drugs were becoming an answer to the decline in drug approvals. Thanks to the Orphan Drug Act (ODA) of 1983, therapies for rare diseases had several advantages. An orphan designation was like the FDA seal of approval as a potentially viable new drug. Grants could be obtained from the FDA to clinically test the drug. The eventual market approval of the orphan designated drug had the special benefit of seven years of exclusivity and tax advantages. This gives a company time to develop networks of parents and doctors in the rare disease community.

Rare diseases are defined as those affecting fewer than 200,000 people in the United States, or affecting more than 200,000 people in the United States, but for which there is no reasonable expectation that sales of the drug treatment will recover the costs. There are about 7,000 rare diseases affecting 25-30 million people in the U.S.

or approximately 1 in 10 Americans. The ODA was passed to provide economic incentives to promote orphan drug research and development. From 1983 to 2010, a total of 3,393 applications for orphan drug designation were submitted, with 2,308 applications receiving designated orphan drug status. Of these 334 became approved drugs for marketing.

At the Center for Rare Disease Therapies (CRDT), we believe that more applications will lead to more designations and in turn will increase the number of market approvals. Therefore, it is our mission to increase the number of orphan drug applications. We do this through education and hosting workshops with the FDA. Obviously, the relatively small numbers of approvals indicates there are many difficulties in reaching approvals. The small number of patients who can be tested is a major factor. It may take years to reach the appropriate sample size to reach statistical power requirements. Once approved and marketed, and despite small numbers of patients, several companies have shown that profits can be made and patients can be served. Gross profit margins of over 80% are reported in the rare disease industry. That is in stark contrast to the 16% pharmaceutical industry average.

The compound annual growth rate of the rare orphan drug market is 5.7% and currently about \$100 billion.

REVENUES FROM SOME ORPHAN DRUGS IN 2010

DRUG	COMPANY	DISEASE	REVENUE
Gleevec	Novartis	CML	\$4,500M
Gleevec	Novartis	GIST	\$4,500M
Sprycel	BMS	CML	\$576M
Fabrazyme	Genzyme	Fabry's	\$188M
Cerezyme	Genzyme	Gaucher's	\$720M
Myozyme	Genzyme	Pompe's	\$412M
Tracleer	Actelion	PAH*	\$1808M
Alduarzyme	Biomarin	MPS I**	\$166M

* Pulmonary Arterial Hypertension **Mucopolysaccharidosis I

Genzyme has been the leader in the fiscal model for high profits, but other companies have been more reasonable, like Sigma Tau and BioMarin. Shire, formerly a specialty company, has expanded since 2005 into orphan drugs and become a global company. Big companies like Johnson and Johnson, Merck and Novartis have established products in the rare disease market, while GlaxoSmithKline and Pfizer have recently created units to increase research and development of drugs for rare diseases.

Novartis has already demonstrated how its orphan drug Gleevec, which was meant to target 9,000 patients with chronic myelogenous leukemia (CML), has been profitable because the drug has saved 9,000 lives per year since 2001 and the population of patients needing the drug to stay alive has grown proportionally. Gleevec was equally good as a therapy for gastrointestinal tumors (GIST), expanding the total patient population number using the drug to 120,000 over 12 years.

At KGI we are pleased to help increase orphan drug applications to the FDA. We are an academic unit working to increase therapies for rare diseases. As an academic unit we are different from bio/pharmaceutical companies as we are not-for-profit. We are also different from patient advocate organizations because we are not bound to a particular disease group. However, our joy has been to help patient advocate groups who have developed their own therapies for their own disease or for someone in their family.

Chris Hempel is the mother of Cassi and Addy, identical twins suffering from Nieman Picks Disease Type 1(NPC-1). Her bright and beautiful babies

gradually began to slow in development of speech and movement. NPC1 is caused by mutations in the NPC1 gene. Lack of the gene causes a buildup of cholesterol in neurons and other cells, resulting in brain degeneration that's referred to as "childhood Alzheimer's." Their determined mother came to a KGI-hosted FDA workshop and received an orphan drug designation for cyclodextrin. The treatment is currently being used intravenously, and approval has been given for intracerebral delivery to make it more effective.

Daniel Darvish and his brother, Babak, are physicians and also patients. They have a rare form of muscle wasting called hereditary inclusion body myopathy (HIBM). It begins in young adulthood and can lead to very severe disability within 10-20 years. It appears in clusters of hereditary linked patients of Middle Eastern, Jewish and Japanese heritage. Daniel Darvish has worked on two potential treatments for his disease. He also came to the KGI-FDA workshop. Both of his therapies were approved, and he and his brother are pursuing clinical testing. KGI professors Jim Osborne and Craig Adams, who operate the Center for Biomarker Research at KGI, are working with CRDT on biological markers of HIBM to demonstrate that treatments are working.

Now with the addition of Dr. Tim Coté to our faculty working on rare diseases, the Center for Rare Disease Therapies is expected to expand its national and international influence to increase life-changing therapies to those in need.

By M. Ian Phillips and Vikram Khanna

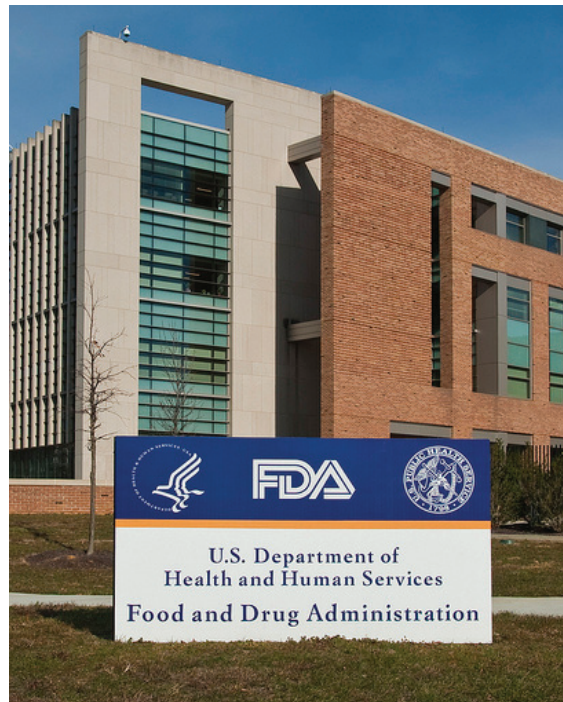
Internships

The Center For Rare Disease Therapies has played an important role in supporting student internships in regulatory affairs through generous grants from the Kenneth T. and Eileen L. Norris Foundation. Students got the valuable opportunity to intern at the Food and Drug Administration (Office of Orphan Products Development) and the Pharmaceutical Research and Manufacturers of America to get first-hand information of how the government and the industry functions in bringing safe and effective products to the market. Some of the students who interned at these organizations were:

- Food and Drug Administration (Office of Orphan Products Development)
 - Will Raasch (2008)
 - Sonali Talele and Aditya Kelkar (2009)
 - Daniel Lev and Chandana Thorat (2010)
 - Laura Wilson, Rudy Tsay and Mimi Ngyuen (2011)

Publications

1. What Orphan Drug Act has done lately for orphan drugs for children with rare diseases: A 10-Year Retrospective Analysis. Chandana Thorat, Kui Xu, Scott N. Freeman, Renan A. Bonnel, M. Ian Phillips, Menfo A. Imoisili, Francesca Joseph. Pediatrics (accepted 2011)
2. Therapies for inborn errors of metabolism: what has the orphan drug act delivered? Talele SS, Xu K, Pariser AR, Braun MM, Farag-El-Massah S, Phillips MI, Thomp-son BH, Coté TR. Pediatrics. 2010 Jul;126(1):101-6.
3. Orphan products: an emerging trend in drug approvals. Timothy Coté, Aditya Kelkar1, Kui Xu, M. Miles Braun & M. Ian Phillips. Nature Reviews Drug Discovery 9, 84 (January 2010) | doi:10.1038/nrd2546-c1
4. Benefits of the Orphan Drug Act for Rare Disease Treatments. Boris Gites, Mona Chughtai, Jessica Chang, M. Ian Phillips. TuftsScope Journal of Health Ethics and Policy, Spring 2010, Express Online.



Faculty

- Timothy R. Coté, MD, MPH - Professor of Practice, Regulatory Affairs
- M. Ian Phillips, PhD, DSc, FAHA – Norris Professor of Applied Life Sciences; Director, Center for Rare Disease Therapies
- James Osborne, PhD - Robert E. Finnigan Professor of Applied Life Sciences; Director, Center for Biomarker Research
- Craig W. Adams, PhD - Research Associate Professor; Director of the Team Masters Project; Assistant Director, Center for Biomarker Research
- Steve Casper, PhD - Henry E. Riggs Professor of Management; Director of the Postdoctoral Professional Masters Program

Supporters

- The Kenneth T. and Eileen L. Norris Foundation
- Sigma-Tau Pharmaceuticals
- Pharmaceutical Research and Manufacturers of America (PhRMA)
- The Gross family

Year 2011-12

OCTOBER



October 11 - 13, 2011

US Conference on Rare Diseases and Orphan Products

Washington, DC

NOVEMBER



November 2 - 3, 2011

Write an Orphan Drug Designation Workshop

Lansdowne, VA



November 10 - 11, 2011

DIA/EMA/FDA Orphan Drug Workshop

London, United Kingdom

November 14 - 15, 2011

Key Opinion Leader & Stakeholder Management in Specialty & Orphan Therapeutics

Miami, FL

November 15 - 17, 2011

2nd Annual World Orphan Drug Summit

Boston, MA



November 29 - December 1, 2011

2nd Annual World Orphan Drug Congress, Europe 2011

Geneva, Switzerland

FEBRUARY



February 4 - 6, 2012

International Conferences for Rare Diseases and Orphan Drugs

Tokyo, Japan



February 28 - 29, 2012 (tentative)

FDA Workshop - "Write an Orphan Drug Designation"

Keck Graduate Institute, Claremont, CA



February 29 - March 2, 2012

International Congress on Research of Rare and Orphan Diseases - RE(ACT)

Basel, Switzerland

APRIL



April 10 - 13, 2012

The World Orphan Drug Congress, USA 2012

Washington, DC



FDA Must Codify Flexibility for Rare Diseases, Coté Says

Reprinted with permission from *The Pink Sheet*, July 25, 2011



Tim Coté

FDA needs to formalize the flexibility it has employed in its rare disease program, according to Tim Coté, the National Organization for Rare Disorders' new chief medical officer and former head of the agency's orphan products development program.

FDA holds orphan products to the same efficacy and safety standards as major market drugs. But it also has a history of understanding orphan drug clinical trials are qualitatively and quantitatively different.

The agency does not always insist review packages include the same amount of data as a product targeting a large patient population, Coté noted.

He said NORD wants the agency to acknowledge in its written policies that rare disease products and reviews are special and usually require a different type of consideration.

"The [review] for rare diseases requires a special level of flexibility and is less amenable to tightly constrained rubrics for study conduct and study review than for common diseases," Coté said in an interview shortly after joining NORD.

"What we'd like to see is the agency's experience – which has [a] record of being flexible – brought into a policy that recognizes that, which it doesn't have right now."

A formal policy should create greater consistency within the review divisions when it comes to rare disease applications. Coté said FDA staff should be trained on it as well.

"Reviewers need to know that and it needs to come from the top and it needs to permeate throughout the institution," he said.

The agency already intends to conduct reviewer training sessions targeted at rare diseases to help teams better understand the diseases and drugs. Industry and FDA agreed to devote user fee revenue to the program as part of the reauthorization of the Prescription Drug User Fee Act.

Advocates Pushing For More Flexibility

The acknowledgement that FDA already is flexible in its treatment of orphan drug applications is interesting given that many rare disease advocates and drug sponsors pushed for more agency flexibility during a 2010 public hearing about rare disease drug review and regulation. Several speakers called for allowing the use of more biomarkers and surrogate endpoints and argued the agency should accept more clinical trials not designed in the standard double-blind, placebo-controlled model.

A report detailing FDA's comments on the opinions expressed at the hearing that could include program improvements still is undergoing

internal clearance, Christine Mueller, OOPD medical officer and outreach coordinator, said July 12. The report was due to Congress in March.

Some drug developers and advocates also called for an Orphan Drug Act renovation that would speed reviews and lower development costs.

John Crowley, CEO and chairman of Amicus Therapeutics, said additional marketing exclusivity will not increase investor interest in the rare disease space, but a speedy and inexpensive application review could attract more venture capital.

NORD also recently decided it would not push for Orphan Drug Act changes in part because it could open the program to unwanted changes.

The group decided it was better to push for improved incentives for drug development within the existing system.

“I Always Wanted To Be an Academic”

Coté stepped down as director of the agency’s Office of Orphan Products Development on May 30 after nearly 22 years in government, joining NORD on July 1. He retired from the Public Health Service Commissioned Corps in 2010 and was in a temporary position as a civil servant while searching for a new job.

Before joining FDA, Coté worked at the National Cancer Institute and Centers for Disease Control and Prevention, where he directed HIV/AIDS, malaria and avian influenza programs as country director for Rwanda.

Debra Lewis, associate director in the Office of Special Medical Programs, will serve as OOPD interim director until the agency hires a replacement. A search already is under way.

Lewis was OOPD’s deputy director during Coté’s administration. FDA said it did not plan any organizational changes because of the departure.

Coté will be splitting his time equally between his responsibilities for NORD in Washington D.C. and the Claremont, Calif.-based Keck Graduate Institute, where he will be a faculty member.

Keck offers bioscience-focused master’s and PhD programs that train students in product development, clinical affairs and informatics, among other subjects. Coté worked with Keck’s Center for Rare Disease Therapies while at FDA, conducting workshops to teach students

how to write orphan drug designation applications, the school said in a written statement.

Those student exercises led to the creation of OOPD’s workshop series with biotech companies and academics on the same subject. FDA had conducted several workshops around the country and recently completed its first session outside the U.S.

Part of his teaching duties will include leading the school’s team masters project, which allows a small group of students to conduct research for industry.

“I always wanted to be an academic to tell you the truth,” Coté said. “Coming to Keck gives me that opportunity to be a real honest-to-God prof.”

Academics Could Improve Reviewer Retention

Creating a more academic culture at FDA itself could help rare disease reviews, Emil Kakkis, president of the Kakkis Every Life Foundation, has said.

In an effort to both improve reviewer retention and expertise, Kakkis said FDA should lessen reviewer workloads and require they teach and conduct clinical work at the National Institutes of Health.

The academic portion would allow reviewers to stay up-to-date with current science and ultimately improve reviews.

Kakkis also has called for centralizing biochemical and genetic disease expertise in an office specializing in reviews for those products.

While FDA senior management seemed supportive of the idea, it does not appear to have gained much traction.

“The challenge is funding an environment where reviewers have the time to engage and be active academically, and also conduct more specialized reviews,” Kakkis said in a July 20 e-mail.

*By Derrick Gingery
The Pink Sheet*

President Schuster Joins National Rare Disease Advocacy Board



KGI President Sheldon M. Schuster, PhD, has been elected to the National Organization for Rare Disorders (NORD) Board of Directors, joining other noted leaders in the rare disease community.

NORD was instrumental in the passage of the Orphan Drug Act of 1983, which provides incentives to pharmaceutical companies to develop drugs for the thousands

of identified rare diseases that affect nearly 25 million Americans. The incentives are crucial since financial profit in developing a drug for a disease that may afflict less than 100 people tends to be quite low.

“Thanks to NORD, there are treatments for literally hundreds of diseases that probably would not have been developed without their work,” Schuster said. “It’s an organization with a very special mission, and it’s great to be a part of that.”

Schuster said one reason he agreed to serve on the board was because NORD and KGI’s Center for Rare Disease Therapies, which is devoted to finding drug therapies for rare diseases, have common goals.

“They are a lobbying organization and we’re an educational institution. We don’t compete in any way but we have a very common and overlapping interest in rare disease therapies,” Schuster said.

Schuster is one of five new members of NORD’s board, which plays an important role in furthering advocacy, education, research and patient services for individuals and families affected by rare diseases.

Tim Coté *continued from page 1*

Coté began his association with the Center for Rare Disease Therapies four years ago when he and his staff started conducting a series of workshops for students on how to write an orphan drug designation application.

What started out as an academic exercise for KGI students led to a series of two-day workshops in which FDA officials met with pharmaceutical and biotech companies, academics and patient advocates and walked them through the orphan product application process step-by-step. The first industry workshop, held at KGI in February 2010, resulted in 18 applications being filed.

The workshops, designed to simplify and demystify the orphan designation application process, were a historic step for the FDA and reflected Coté’s desire to interact with academia and develop partnerships to benefit people with rare diseases. It was the first time that the FDA had sent so many people outside Washington, DC, to assist companies and individuals in preparing orphan drug designation applications.

Ian Phillips, PhD, KGI’s Norris Professor of Applied Life Sciences and co-director of the Center for Rare Disease Therapies, said, “Dr. Tim Coté is highly respected in the rare disease community and brings enormous stature, experience and connections in the orphan drug and FDA regulation fields. With his reputation, teaching ability and knowledge, I look forward to working with Tim to elevate the Center for Rare Disease Therapies to a higher level.”



Center for Rare Disease Therapies
KECK GRADUATE INSTITUTE
of Applied Life Sciences