Dr. Rob KOTIN

Seeing the Big Picture in Gene Therapy

DRIVEN TO INNOVATE BY CURIOSITY

AFFABLE. ICONOCLASTIC.

Dr. Rob Kotin is one of the most influential thought leaders in gene therapy today.

One of the most influential thought leaders in gene therapy today, Rob Kotin, Ph.D.'s work has the potential to make a lasting impact on the lives of patients suffering from debilitating diseases of the central nervous system. His basic science contributions have influenced all aspects of adeno-associated virus-based (AAV) gene therapy.

Dr. Kotin has the ability to see the big picture and is not held back or restricted by existing dogma.

During his 28-year involvement in virology, he made several discoveries that are notable. In 1988 (published in 2000) he discovered that the adeno-associated virus virion DNA integrated into a defined region on human chromosome 19 that was designated AAVS1. Among the DNA viruses that infect animals, the phenomenon of preferential integration is unique to AAV. AAVS1, the integration locus, has been proven to be a "safe harbor" for exogenous genes and other companies have targeted this site to produce modified cell lines.

In 1998, Dr. Kotin's laboratory discovered that AAV DNA can replicate in invertebrate cells and developed a scalable process for producing rAAV using the baculovirus/Sf9 cell system. During the 2000s, they then improved and optimized the process. Several companies licensed the materials and methods for producing rAAV in the invertebrate cells. Glybera, the first AAV gene therapy product to receive regulatory agency approved for human use, was developed with materials and processes licensed from his laboratory.

His development of methodology for large-scale production of AAV in baculovirus has the potential to change the types of diseases that gene therapy can address, and allow for treatment of systemic disseminated diseases rather than localized ones in specific organs.

He has also made many contributions to understanding the role of rep in AAV integration, rep's ability to affect the cell, and demonstrating that AAV capsids have unique tropisms.

Many believe AAV is a critical element in the success of gene therapy, and those who have worked with him say Dr. Kotin's background in the fundamental biology of AAV vectors will be instrumental to the future of gene therapy.

Dr. Kotin recently accepted a job as VP of virology at Voyager Therapeutics, and notes that the challenges of establishing a research vector facility, a cGMP manufacturing facility, and conduct basic virology R&D has been daunting.

"However, with support of the Voyager Therapeutics team, we have overcome one challenge after another," he says.

Gene therapy relies on a gene target, as well as safe and effective delivery of a genetic modulator of that target. The latter requires a thorough knowledge of, and experience with, the delivery vehicle, in Voyager's case AAV, and a perspective to develop it as a pharmaceutical entity. Those who have worked with him say Dr. Kotin has the know-how, experience, and perspective, and he is ideally positioned to lead Voyager toward these goals and the development of therapies for serious CNS disorders, including ALS and Friedreich's ataxia.

Dr. Kotin's work in the area of gene therapy has led to his strong support for organizations that are striving to help people with such debilitating conditions, including the Duchenne muscular dystrophy foundations, Friedreich's Ataxia Research Alliance, and the Michael J. Fox Foundation.

Science in general inspires Dr. Kotin, who says perhaps he will become a naturalist in his next career move.

"Very little is known about speciation processes even after 150-plus years since Darwin's On the Origin of Species," he says "Modern molecular biology tools and powerful super-computers can run modeling and simulation programs that may revolutionize our understanding of how species evolved. Undoubtedly a romanticized version of a naturalist, wandering through remote habitats to collect new specimens, discovering new species, etc., is not realistic, but it in some regards, such a lifestyle, maybe not a career, is feasible."

Dr. Kotin encourages people to think outside the box and to not be constrained by the current thinking on a topic.

And he influences many individuals in the field by his smart, yet laid back demeanor and his focus on a key aspect of this technology for academia and industry. PV

Getting to Know...

Robert Michael Kotin, Ph.D.

TITLE: VP

COMPANY: Voyager Therapeutics

EDUCATION: Ph.D., M.S., Rutgers University; B.A., University of California

FAMILY: Wife, Charlotte McGuinness, J.D., Ph.D.; children, Stevan and Cordelia

HOBBIES: Squash, bicycling, crossword puzzles

BUCKET LIST: Learn French or Italian; learn a musical instrument; learn how to draw and paint

SOCIAL MEDIA:

Dr. Dinah SAH
Driving Scientific Innovation

DETERMINED. DEDICATED.

Dr. Dinah Sah has been a driving force in innovative discovery methods to uncover novel therapies for the past 20 years.

With 25 patents and several novel drugs in development in her wake, Dr. Dinah Sah has been a driving force toward innovative discovery for novel therapies for the past 20 years. Just this year, she has taken on a new assignment as senior VP of neuroscience of Voyager Therapeutics, a gene therapy company focused on diseases of the central nervous system. Colleagues and former coworkers say they are confident that Dr. Sah will bring success to the start-up company, just as she has in her other endeavors.

A pioneering force behind the development of novel drug classes that have the potential to treat devastating neurological disorders, her passion and conviction for innovative approaches led to the landmark demonstration in 2011 of human proof-of-mechanism for a new class of therapeutics based on RNA interference (RNAi). Bringing this same commitment to innovation in drug discovery to Voyager Therapeutics, she will focus on developing a class of therapeutics based on adeno-associated virus (AAV) gene therapy, with breakthrough potential for the treatment of CNS neurodegenerative diseases. In just her first few months at Voyager she has been instrumental in driving the selection of its lead product programs, based on extensive analyses of multiple aspects of the technology platform and potential disease applications and molecular targets.

"I am motivated and inspired by the potential to discover and develop a transformative drug for neurodegenerative diseases," Dr. Sah says. "I lead by example — a demonstration of dedication, commitment, hard work, and doing the right thing, with high standards for all activities."

Before joining Voyager, Dr. Sah spent seven years at Alnylam Pharmaceuticals, where she was most recently VP of research, providing scientific leadership and administrative oversight of discovery research and multiple research and development programs.

Before Alnylam, Dr. Sah was associate director of research at Biogen, where she led neuroscience research and strategic planning for neurobiology, and before that, she headed neuroscience research at Signal Pharmaceuticals, where she also led multiple corporate partnerships and projects.

Dr. Sah is an inventor and holds more than 25 patents, and her publications across diverse research areas include 18 articles in the New England Journal of Medicine, Nature Medicine, Nature Biotechnology, Nature Neuroscience, Nature Chemical Biology, Nature Reviews Drug Discovery, Molecular Therapy, Neuron, PNAS, and EMBO Journal.

DRIVEN TO INNOVATE BY
POTENTIAL

Dr. Sah is not afraid of a challenge and is relentless in her efforts to overcome obstacles related to therapeutic development. The programs that she oversaw at Alnylam are her legacy, colleagues say. They are either in Phase II or starting Phase III clinical trials; a testament to her dedication to bringing novel therapeutics forward for patients. Her project for neuropathic pain at Biogen is now in Phase II trials. Dr. Sah was the main driver of this project, and it would have been canceled, says one colleague, if not for Dr. Sah's ability to positively influence Biogen's management team.

Science is her passion both in and out of the lab, and she has spent many hours involved with increasing the presence of science in the Caribbean for both educational and economic reasons through two nonprofit organizations: the Caribbean Science Foundation (CSF) and the Caribbean Diaspora for Science, Technology and Innovation (CADSTI).

Her husband, Dr. Cardinal Warde, professor of electrical engineering at MIT, established CSF in 2010. Its mission is to assist with the diversification of the economies of the Caribbean region by harnessing science and technology for economic development, and to help raise the standard of living.

Specifically, the CSF plans to stimulate technology-based entrepreneurship by identifying and funding science and technology projects in new and existing enterprises that are relevant to the economic development needs of the region. Dr. Sah serves on the governing council for CSF. CSF also facilitates a flagship program, SPISE, a 24/7, four-week immersion program for promising Caribbean high school students interested in pursuing a career in STEM, which Dr. Sah co-directs. CADSTI is an international body of professionals who have an interest in the development of the Caribbean region.

CADSTI recognizes that there is a vast talent pool within the larger Diaspora whose skills go untapped by the Caribbean community. One of its goals is to facilitate the networking that will bring resources from the Diaspora to the region for the mutual benefit of all parties. Dr. Sah serves as president of the newly formed New England chapter of CADSTI, created to secure funding to bring Caribbean students to the United States for additional training and experience before applying for college.

Through the SPISE program, Dr. Sah has influenced many young students, who may one day become industry thought leaders, with the ability to bring innovative new businesses to the islands of the Caribbean, which could shape the region to be technology-driven instead of tourist-driven.

Dr. Sah says it is very rewarding to provide guidance to talented young people and facilitate their career development.

According to colleagues, Dr. Sah not only possesses in-depth scientific and drug discovery and development expertise, but a thoughtful leadership style and a high standard for performance, both in quality and quantity, which inspires others to do the same. As a result, she carries the universal respect of her professional colleagues at all levels, and creates a culture of excellence. PV

Getting to Know...

Dinah Sah, Ph.D.

TITLE: Senior VP, Neuroscience

COMPANY: Voyager Therapeutics Inc.

EDUCATION: Ph.D., Neurobiology, Harvard University; B.S., Biology, MIT

FAMILY: Husband

HOBBIES: Tennis, classical music

CARING.
TENACIOUS.

Dr. Boaz Mendzelevski's many achievements have made him an asset to not only the companies he has worked for but to the field of cardiac safety as a whole.

Dr. Boaz MENDZELEVSKI

At the Heart of the Matter

Over the last 20 years, Boaz Mendzelevski, M.D., has dedicated his energy to advancing the field of cardiac safety, helping to drive new therapies from both an R&D and a regulatory perspective. He is a pioneer and advocate for cardiac safety and has had a large hand in shaping this evolving field to where it is today. Dr. Mendzelevski's research and focus on drug-induced QT prolongation and pro-arrhythmia risk supported the first cardiac safety regulatory guidance — the 1997 EMEA CPMP/986/96 Points to Consider.

Dr. Mendzelevski was invited by the FDA and ICH to serve on the ICH-E14 (Step 3) expert advisory panel, where he contributed his cardiology expertise toward the development of this important regulatory guidance, which has been adopted by U.S., E.U., and Japanese regulatory agencies. The guidance requires sponsors to assess ECG QT intervals using a formal TQT study. These studies are important for identifying potential pro-arrhythmia risks associated with treatments in development. However, some of these studies, in particular the parallel group studies, require hundreds of subjects to achieve the specified regulatory endpoints for a Phase I study.

Dr. Mendzelevski and colleagues, in collaboration with the FDA QT-Interdisciplinary Review Team (IRT), have explored, pioneered, and validated a novel TQT study design that enables a significant reduction in sample size and satisfies existing regulatory requirements for demonstrating assay sensitivity and cardiac safety endpoints. This new study design offers significant cost savings for sponsors and reduces the logistics of running large TQT studies, enabling smaller clinical pharmacology units to participate in such studies.

Dr. Mendzelevski continues to educate sponsors and clinicians to this novel design and is a co-author on a recent peer-reviewed publication outlining this successful approach for TQT studies. In addition, Dr. Mendzelevski is the first internationally recognized cardiac safety expert to work with some of the leading Asian regulatory agencies, primarily the Japanese PMDA and the Chinese C-FDA, in implementing ICH-E14 standards in Asia.

DRIVEN TO INNOVATE BY

PASSION

He has been invited to attend and lecture at the Peking University Chinese Course in Drug Development and Regulatory Science (CCDRS), a novel, custom-designed, advanced university course that provides an understanding of the biopharmaceutical R&D process and covers the key aspects of lifecycle management for new therapeutic products. He has introduced cardiac safety into the curriculum of this course and values the opportunity to educate the next generation of pharmaceutical, academic, and regulatory leaders in China.

Dr. Mendzelevski has initiated and led most of the global Annual DIA Cardiac Safety (CS) Conferences in Europe since 2006, in Japan since 2010, in China since 2014, and supported the U.S. DIA CS events since 2009.

With a deep understanding of the basic concepts of cardiotoxicity associated with cancer therapies, Dr. Mendzelevski is also one of the first pioneers to develop and promote the concept and practice of cardio-oncology, a novel discipline aimed at identifying and mitigating the cardiac toxicity of oncology drugs.

He recently published on this topic in the journal of Expert Opinion in Drug Safety and will co-chair an upcoming DIA Cardio-Oncology conference. Additionally, he is a member of the International Cardio-Oncology Society (ICOS) writing group that is currently developing a clinical practice consensus paper.

Among his many industry achievements, Dr. Mendzelevski started Cardiac Alert Ltd., a cardiac safety core lab based in London, and then relaunched it as the Quintiles core ECG lab in Mumbai, India.

Dr. Mendzelevski has authored more than 250 reports in support of NDA regulatory submissions and has recent peer-reviewed publications on the topic of cardiac safety.

He has sat on the advisory boards for many large pharmaceutical companies, including Merck KGaA, Otsuka, OxiGene, Schwarz, Takeda, and Teva and has served on the data safety and data monitoring boards for Novartis, GSK, Schwarz, and others.

Getting to Know...

Dr. Boaz Mendzelevski

TITLE: VP, Cardiology

COMPANY: BioClinica Inc.

EDUCATION: M.D., Ben-Gurion University of the Negev

FAMILY: Married, four children

HOBBIES: Tai Chi, Qigong, reading, hiking, dining, socializing, and traveling

BUCKET LIST: Space travel, flying a light plane, parachuting, traveling to exotic places

ASSOCIATIONS: Drug Information Association (DIA); Safety Pharmacology Society (SPS); International Cardio-Oncology Society (ICOS); Heart Rhythm Society (HRS)

SOCIAL MEDIA:

Today in his role as VP of cardiology at BioClinica, he contributes his scientific expertise and innovative strategies to the company, while also providing his insights as an important subject matter expert for the company.

Dr. Mendzelevski has made some interesting observations about the world of medicine compared with industry. As a physician, he was taught that the ultimate responsibility for his patients lies with him and that he should trust only himself when it concerns his patients' lives and well-being.

"When I joined the industry I learned that the task at hand is bigger than any one person and that we can only succeed by working as a team and trusting each other," he says. "This was a major insight and a huge mental shift for me in moving from the clinical medicine environment where you are responsible for one patient at a time to pharmaceutical medicine where you treat hundreds or thousands of patients in a single study program."

He motivates those he works with by sharing, educating, inspiring, and empowering, and he seeks to inspire primarily by sharing knowledge and passion, and providing a personal example.

In giving back, Dr. Mendzelevski and his family support five different children's charities and two animal charities.

"We believe that deprived children, everywhere, deserve better chances for education and a good future," he says. "Investing in the future of young members of society will not only improve their chances for a better life, but will also benefit society as a whole. We also love animals, we have a Labrador, and do our best to support animal charities and shelters." PV

Dr. Kate DAWSON

Grace Under Fire.

CALM. DRIVEN.

Dr. Kate Dawson is passionate about creating treatments for neurological disorders so that patients can live better with their disease.

DRIVEN TO INNOVATE BY PATIENTS

The database locked on April 1, 2010, and a week later people couldn't believe their eyes. The Phase III data on Tecfidera were in, and the news was very good. The Phase II data had proven that the drug worked on brain lesions, had good safety and tolerability profiles, but it looked like it would be only similar in efficacy to approved first-line therapies. Team members were expecting positive results to some degree, but not as good as what they saw.

Even Dr. Kate Dawson, VP of global medical neurology at Biogen Idec, the person whose unfaltering dedication was pivotal to the success of the program, couldn't believe the results.

"I was optimistic that we would see results better than what we saw in Phase II, but even in my wildest imagination, I never expected to see the results we had," she says. "I remember the moment well when we all realized that we had a drug that could be a very useful therapy and potentially change a lot of people's lives."

Tecfidera is currently the No. 1 prescribed oral treatment for MS in the United States.

Colleagues say this achievement wouldn't have happened as smoothly, efficiently, and quickly without Dr. Dawson at the helm.

Her team spirit and enthusiasm, as well as calm demeanor, led the company through this daunting task. Working for days straight at a time, flying to three countries in under 72 hours, and even working on Christmas Day, were all sacrifices she made for her team and her company. According to colleagues, she lives and breathes Biogen Idec's commitment to patients.

Her experience as a physician of neurology — she has been on the faculty at Massachusetts General Hospital for a number of years — has given her a greater understanding of what people with neurologic diseases, including MS, experience on a daily basis.

Dr. Dawson chose to move from practicing medicine to working at a biotech company so that she could impact the lives of more patients.

"In moving from academia to a company such as Biogen Idec, my hope is to be able to bring a therapy to many patients," she says. "Lucky for me, Tecfidera exceeded all expectations, so it's been really great to know that I'm helping even more patients than I thought I would."

Tecfidera is not Dr. Dawson's only achievement at Biogen Idec, although it may become her legacy. She has played a significant role in the transformation of Biogen Idec over the last few years, helping to evolve it from a modest biotechnology company to a global leader in the fight against MS.

Dr. Dawson looks forward to growing the global medical neurology group, to prepare for the potentially new both MS medications that are in the pipeline and to expand the focus to other areas of neurology.

"One of the big challenges and something that is on my professional agenda is to grow and scale the company as a global medical organization as we grow within the neurology therapeutic area," she says. "As a company, we're moving from being a MS-specific neurology company to one that addresses neurology needs on a wider spectrum."

When she is not navigating the drug filing process and remaining calm under fire, Dr. Dawson prefers reading books to Nooks, and one book in particular was instrumental in starting her on her career in neurology. As a sophomore in high school, she read The Brain by Dr. Richard Restak.

"It was the first time I had really understood how the brain works, and all the really interesting things about our brains, and consciousness, and that was the start of my love story with neuroscience." PV

Getting to Know...

Dr. Kate Dawson

TITLE: VP Global Medical Neurology

COMPANY: Biogen Idec

EDUCATION: B.S., Columbia College; M.D., Albert Einstein College of Medicine; Neurology Residency and Fellowship, Massachusetts General Hospital

FAMILY: Husband; son; crazy dog and a cat

HOBBIES: Travel and reading

BUCKET LIST: Travel with family to Australia; have a second home in Sante Fe

AWARDS/HONORS: Biogen Idec Grace Under Fire Award, Innovation Award, and MAXA (Management And eXecutive Academy)

SOCIAL MEDIA:

EMPOWERING. MOTIVATING.

Krista Payne has found satisfaction in leading winning teams to greatness.

Krista PAYNE

The Forest for the Trees

DRIVEN TO INNOVATE BY SATISFACTION

Krista Payne is a vital member of the UBC team — often described as the spark plug — and a key partner to pharmaceutical manufacturers, due in part to her contributions to observational research and her ability to lead and inspire.

Ms. Payne leads a multidisciplinary team of experts who combine significant scientific expertise in areas such as health economics, outcomes research, epidemiology, and registries, along with advanced technical and operational support, to provide full-service, high-quality evidence-gathering services.

She and her team design observational studies that demonstrate the economic or humanistic value of medicines or devices in support of market access. These studies are increasingly important, both in the United States and abroad, as the evidence needs of payers and regulators converge.

At a time when so much of pharma's attention is directed at building a compelling value story for payers, Ms. Payne sits at the head of a pivotal department at UBC. Colleagues say she walks both sides of the fence that separates clinical from commercial beautifully, helping clients think beyond the traditional to embrace the innovation that's driving the market access strategies of tomorrow.

Ms. Payne began her work in the industry with a small research department that was part of a UBC acquisition.

Ten years ago, the Canadian-born former high school guidance counselor launched UBC's health economics and values demonstration services and under her leadership it has continually grown. To celebrate this milestone, her team gave her a framed print by Tom Thompson, an early 1900s Canadian artist from the Group of Seven. The illustration shows the beauty of the Canadian forest and is a reminder of a lesson she teaches: don't lose sight of the forest while tending to the trees.

"In this fast-paced industry, the demands are high, and it's easy to lose perspective, especially if we lose touch with fundamental goals and objectives, she says. "To date, my most valued achievement has been this very personal recognition I received from my staff. There is no greater honor than that delivered by whom you serve."

Over the years, Ms. Payne has introduced innovations in the design of real-world studies, including retrospective chart review studies, time-and-motion studies, pragmatic trials, and epidemiological observation studies. Innovative approaches go beyond traditional site-based data collection, leading to increased efficiencies, and involving cross-functional collaborations among departments.

Her strong pharmacoeconomic background has proven instrumental in understanding pharmaceutical and biotech clients' needs for economic evidence to support the value of existing and new products. Ms. Payne has an outstanding ability to help translate the evidence needs into the right design.

Ms. Payne and her team have specialized in designing tailored solutions to transform primary outcomes resulting from observational studies into economic value messages. Scientific integrity and robust methods are key to her work, though all studies she designs integrate practical and operational considerations as well. Ms. Payne and her team have successfully lead numerous retrospective chart review and other studies in more than 15 countries around the world. These studies have varied in scale from fewer than 10 sites to more than 300 sites, with patient cohorts ranging from a few hundred to thousands.

In addition to her scientific skills, she is also a mentor to those around her professionally and a valuable member of her community where she has been a published lobbyist for mental health care policy reform. Over several years of acquisitions and organizational change, Ms. Payne led her team through this turbulence with grace.

As a mentor, Ms. Payne makes herself available to many, sharing her knowledge and expertise. She has a giving heart and practices what she preaches — collaboration, honesty, and encouragement. As a manager, she always acts with integrity and always gives credit to her staff and makes time in her super-busy timetable to really listen to them. She will make time for them even if they are based an ocean apart.

"There are no solo acts in this fast-paced environment, and successful leaders are those who can build and showcase high-performance teams," she says. "As leaders, sometimes we just need to stay out of the way until we're asked to step in. By stepping back, we give others the chance to demonstrate what they can do."

Recognizing when to step in and when to step back is not always easy but Ms. Payne says she tries to motivate her staff to think outside of the box and push their own limits for personal and business benefit. When they do achieve success in their own right, it's an opportunity for them to shine and inspire others in turn.

"It's that euphoric buzz of success we feel when we know we've delivered and surpassed expectations, that motivates us to jump back in the trenches and start all over the next day," she says. "I couldn't lead in any other way; at my core, I'm a pack animal, so by nature, I win or lose with my team and close colleagues, and in both scenarios it's only heartfelt and worth the journey if I'm feeling connected." PV

Getting to Know...

Krista Payne

TITLE: Executive Director and Principal Scientific Consultant; Value Demonstration; Safety, Epidemiology, Registries and Risk Management

COMPANY: United BioSource Corp.

EDUCATION: B.S., Psychology, Queen's University; Master's, Educational Psychology, McGill University

FAMILY: Wife, Ingrid; son, Jonathan; poodles, Rolo and Milo; cats, Motica and Bébé

HOBBIES: Alpine skiing, soccer, ball hockey, painting, gardening, wine adventures

BUCKET LIST: A private Borneo tour with Jane Goodall; a retreat to Monastic Tibet; ocean-kayaking with Ingrid and Jonathan and a pod of dolphins

AWARDS/HONORS: UBC Circle of Excellence

ASSOCIATIONS: ISPOR, ISPE

SOCIAL MEDIA:

"Success is simple. Do what's right, the right way, at the right time."

– Arnold H. Glasow

Congratulations to UBC's Krista Payne on being named to the PharmaVoice Top 100!

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ENERGETIC. POSITIVE.

Dr. Nancy Dreyer is unwilling to accept the status quo when she can see a way for improvement.

Dr. Nancy DREYER
Innovative Problem Solver

Nancy Dreyer, Ph.D., MPH, has made numerous contributions to the field of epidemiology as a high-level thinker, visionary leader, and true collaborator.

Dr. Dreyer not only challenges those who work for her to lead and take responsibility but takes the time to work through problems with her team in a way that both teaches and solves problems. As her grandmother used to say "you can't put an old head on young shoulders," but she adds that "what we can do is help others learn from our mistakes and theirs."

One of her most challenging roles was leading the creation of a global patient registry for human infection with avian influenza (H5N1). Without any support from international agencies, she and her team went around the world, following media reports of outbreaks, and developed collaborations with individual physicians, health departments, and academics to collect data on human cases.

"We assembled data on two-thirds of the world's known cases from 12 of the 15 countries with known outbreaks," she says. "We discovered that even delayed initiation of treatment with an appropriate antiviral led to a substantial reduction of death from infection with H5N1, and were able to characterize the clinical presentation and treatment effectiveness in both children and adults."

Those who worked with her on the registry note that Dr. Dreyer simultaneously balanced working as both a high-level executive, providing strategy for how the team could successfully implement such a complex, challenging study, as well as a partner and team player. She worked on a detailed level with all members of the team to solve difficult problems and provide mentorship.

DRIVEN TO INNOVATE BY STATUS QUO

A specialist in real-world research, which is used to inform decision-making after medical treatments come on to the market, Dr. Dreyer says the industry is in the strategic position of having growing access to large clinical datasets from healthcare settings, some of which can be used to answer many clinical, payer and policy questions.

"Our challenges relate to understanding why and how things are recorded, and when data are missing, why," she says. "For example, if there is no indication that an expected lab test was performed, was it truly not done or were the data stored elsewhere or recorded in a place we haven't looked? Another important set of challenges relate to prospective data collection and selecting the key data that are essential to the research objective as opposed to other data that would be nice to have data but may be overly burdensome to collect."

Going forward, Dr. Dreyer says the future lies in personalized medicine, and the path requires systematic assembly and evaluation of clinically meaningful information for many subgroups of interest, such as the young and the elderly as well as those with co-morbidities, so clinicians and others who make decisions for health systems are properly informed.

"Evidence-based medicine needs real-world evidence," she says.

Dr. Dreyer's contributions to the field of epidemiology are numerous and globally recognized. These include, but are not limited to, development of the GRACE principles, an ISPOR task force report of use of observational studies to assess comparative effectiveness, senior authorship on the landmark AHRQ publication Registries for Evaluating Patient Outcomes: A User's Guide and Developing a Protocol for Observational Comparative Effectiveness Research.

She is excited by the work she does and the people she works with.

"We have a shared vision to be the best," she says. "Everybody wants to be a winner and likes working with winners."

She draws inspiration from successful entrepreneurs such as Richard Gliklich, who founded Outcome Sciences Inc., and Gerald Chan of the Morningside Group.

"Both are intelligent, inquisitive, well-informed, and take action," she says.

A strong advocate for good education, Dr. Dreyer supports charitable causes that provide educational scholarships at the undergraduate and graduate levels.

"A good education helps to cultivate talent and open doors," she says.

One of her colleague's credits Dr. Dreyer with encouraging her to pursue a master's degree and Ph.D. in epidemiology. PV

Getting to Know...

Nancy A. Dreyer, Ph.D.

TITLE: Global Chief of Scientific Affairs

COMPANY: Quintiles

EDUCATION: A.B., Brandeis University; MPH and Ph.D., University of North Carolina, School of Public Health

FAMILY: Husband, Ken Rothman; three daughters: Emily, Meg, and Samantha

HOBBIES: Experimental cuisine; gardening

BUCKET LIST: Visit the Taj Mahal, see the terracotta army in Xi'an; sail in big water

ASSOCIATIONS: Board member of the Drug Information Association (DIA), International Society of Pharmacoepidemiology, European Network of Centers of Pharmacoepidemiology & Pharmacovigilance (EncEPP)

SOCIAL MEDIA: in

Dr. Pablo UMAÑA
Research Breakthroughs

The father of the first glyco-engineered antibody to reach cancer patients, Pablo Umaña, Ph.D., is one of the most inspiring and committed researchers of the last decade.

Dr. Umaña's belief in his research and continued dedication to glyco-engineered antibodies led to the discovery of a new drug for the treatment of cancer — Gazyva/Gazyvaro.

Dr. Umaña co-founded GlycArt Biotechnology AG in 2001, a spin-off company based on his research around glyco-engineering of antibodies at the ETH-Zurich.

Understanding the glycosylation biology of proteins was one of Dr. Umaña's goals and he was convinced that antibody efficacy could be enhanced by applying this knowledge.

Dr. Umaña and his team developed technologies to change glycosylation patterns of therapeutic antibodies. As chief scientific officer of GlycArt, Dr. Umaña led the discovery and preclinical development of engineered therapeutic antibodies for cancer immunotherapy, including obinutuzumab (GA101).

After the acquisition of GlycArt by Hoffmann-La Roche AG in 2005, he continued leading discovery research at Roche GlycArt, a Pharma Research and Early Development (pRED) R&D site focused on engineered antibodies and cancer immunotherapy.

DRIVEN TO INNOVATE BY PASSION

In 2008, a molecule that was discovered in research at Roche Schlieren in Dr. Umaña's labs astonished scientists. Obinutuzumab induced remarkable tumor shrinkage in a patient with aggressive lymphoma. Until that point nothing else had helped. To see such a dramatic response in someone for whom everything else had failed was extremely rewarding and motivating.

"When we started this six years ago not many people believed that we would be as successful as we ended up being," Dr. Umaña says. "The obinutuzumab project was risky, with an unconventional approach that relied substantially on a pioneering technology."

An additional challenge was that the team was trying to develop a medicine that would be superior to another Roche drug (rituximab) that was already viewed as extremely powerful. The team around Dr. Umaña ambitiously pursued a significant scientific breakthrough with potential to dramatically raise the efficacy bar of current therapies. Such a feat could not have been accomplished without an abundance of passion, unwavering belief, top-caliber science, and steadfast determination. Obinutuzumab represents a new development in the science of monoclonal antibodies directed against B cell malignancies, such as chronic lymphocytic leukemia (CLL) or non-Hodgkin lymphomas (NHL).

It is the first Fc engineered antibody worldwide to be filed for registration with the Food and Drug Administration and European Medicines Agency; it is also the first pRED antibody to be filed for registration.

Dr. Umaña, who is head of discovery oncology and large molecule research at Roche pRED, says after the success of the Gazyva discovery and early development, the challenge for his department was to re-invent itself as an oncology research group pursuing novel, different approaches in the field of cancer immunotherapy.

"It took a lot of hard work and creativity to move beyond what we were comfortable with, while still building on our strengths," he says. "This strategy has now paid off with a rich early pipeline of highly differentiated drug candidates representing more than 50% or Roche's pRED oncoimmunology portfolio."

He is focused on achieving impressive gains in efficacy of cancer treatment, demonstrated in clinical trials, by using combinations of novel drug candidates within Roche's portfolio.

Motivation for Dr. Umaña comes from taking on a worthwhile challenge, and he seeks to motivate his team by presenting a clear vision and engaging team members to realize success as a team. PV

TENACIOUS. PASSIONATE.

Dr. Pablo Umaña is one of the most inspiring and committed researchers of the last decade.

Getting to Know...

Pablo Umaña, Ph.D.

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