Takeda's Innovators IN SCIENCE AWARD

Dr. Adrian Krainer

Dr. Jeong Ho Lee

Takeda Pharmaceuticals and the New York Academy of Sciences are drawing attention to the urgent needs of patients worldwide affected by an estimated 7,000 rare diseases. The 2020 Innovators in Science Award recognizes scientists from around the globe for their exceptional research in rare diseases.

Established in 2016, the Innovators in Science Award is administered by the New York Academy of Sciences and sponsored by Takeda Pharmaceuticals. This global award recognizes a promising early-career scientist's and an outstanding senior scientist's contributions to biomedical science and is intended to support their commitment to innovative research.

Two prizes of $200,000 are awarded each award cycle to winning scientists who have distinguished themselves for the creativity and impact of their research in a designated therapeutic area.

Prof. Adrian Krainer, Ph.D., St. Giles Foundation Professor, at Cold Spring Harbor Laboratory, was named the senior scientist winner. He is recognized for his outstanding research on the mechanisms and control of RNA splicing, a step in the normal process by which the information in DNA is converted into proteins. Prof. Krainer studied a splicing defect in patients with spinal muscular atrophy (SMA), a devastating, inherited pediatric neuromuscular disorder caused by loss of motor neurons, resulting in progressive muscle atrophy and eventually death. His lab elucidated regulatory mechanisms controlling SMN2, a gene that is incorrectly spliced in SMA. Then, in collaboration with scientists at Ionis Pharmaceuti-cals, they developed a novel antisense oligonucleotide that is effective in correcting SMN2 splicing, thus increasing the levels of SMN, a protein required for motor-neuron survival. This antisense oligonucleotide — nusinersen (Spinraza) — was approved by the FDA in December 2016, and subsequently in more than 50 additional countries. Spinraza was the first drug approved for the treatment of SMA, the first approved drug to correct a splicing defect, and the first drug for an inherited neurodegenerative disorder that can delay and even prevent disease onset.

Jeong Ho Lee, M.D., Ph.D., associate professor/KAIST Endowed chair professor, Korea Advanced Institute of Science and Technology, director at the National Creative Research Initiative Center for Brain Somatic Mutations, and co-founder and chief technology officer at SoVarGen, is recognized as this year's early-career scientist. Prof. Ho Lee is recognized for his research investigating genetic mutations occurring in a subset of cells in the body, a phenomenon referred to as somatic mosaicism. Particularly, Prof. Lee studies the genetic mutations in stem cells in the brain that result in rare developmental brain disorders. These rare mutations can cause dysfunction of the entire brain, resulting in epilepsy and tumor formation. Prof. Lee has identified the genes responsible for several developmental brain disorders including focal cortical dysplasia, Joubert syndrome — a disorder characterized by an underdevelopment of the brainstem — and hemimegalencephaly, which is the abnormal enlargement of one side of the brain. Fundamental knowledge of the genetic basis for these disorders in humans is a necessary first step toward the development of potential new treatments and diagnostic tools. His work has also influenced scientific thinking about tumorigenesis and has helped lay the foundation for studying somatic mosaicism in neuropsychiatric and neurodegenerative diseases such as Alzheimer's disease.

WOMAN OF THE WEEK PODCAST SERIES

SEPTEMBER

Eloiza Domingo, Global Head of D+I, Astellas
Tzvia Bader, CEO, TrialJectory
Beenu Kapoor, VP, IT Trial Management, Products Lead, IQVIA
Shonda Anderson-Williams, Healthcare & Life Sciences Digital Transformation Leader, Microsoft
Georgette Pascale, Founder and CEO, Pascale Communications

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PharmaVOICE 100 NEWS

DR ROZ JOINS THERAVANCE

Dr. Roslyn Schneider

Roslyn Schneider, M.D., has been named VP, Scientific, Medical and Patient Affairs at Theravance Biopharma. Most recently, Dr. Schneider started her own consultancy RozMD Patient Affairs Consulting LLC after a 13-year tenure at Pfizer, where she held positions of increasing responsibility, culminating in the role of global patient affairs lead. Dr. Schneider spent 20 years at Beth Israel Medical Center practicing internal medicine, pulmonary, and critical care in academic and clinical settings.

Dr. Schneider is a 2018 PharmaVOICE 100.

2020 Survey Results: PATIENT SERVICES AND PATIENT ENGAGEMENT

▶ The No. 1 job of patient services is to improve the patient experience and use of treatment (initiation, adherence, experience).
▶ Acknowledging the difficulty of consistent high quality from single vendors who do everything, patient services seeks best-of-breed vendors and to establish tighter coordination between these vendors.
▶ The top two priorities for patient services in the coming year are to get patients started post-Rx and to maximize patient satisfaction with treatment.
▶ COVID is accelerating growth in patient services with the greatest investment going to nurses, video, and SMS channels.
▶ Net Promoter Score varied widely across Patient Services vendors. McKesson and Human Care Systems were highest with scores of 7.1 and 7.5, respectively.

Source: Human Care Systems

2020 PharmaVOICE 100 Celebration

PharmaVOICE 100 24-Hour Celebration Marathon

PharmaVOICE, like the rest of industry, had to find a way to pivot our business and strategy around our annual in-person PharmaVOICE 100 event, when the world was struck by the COVID-19 pandemic. We felt it was our mission to find a way to celebrate the individuals who are striving tirelessly to find answers for patients, care givers, in essence humanity, to solve the biggest healthcare issues around the world. In true PharmaVOICE fashion, we wanted to pave the way and make a bold — some might say crazy — statement in response to our call for the industry to raise the bar — the theme that resonated throughout the event.

Many asked why 24 hours? Why not 8 hours? For us the answer was easy. We wanted to engage our global community of PharmaVOICE 100 honorees, thought leaders, readers, and sponsor companies in a way we never could with a live event — after all, we are living in a new virtual reality.

We hear everyone talking about the next normal, but for months now we have been asking why go back to normal when we have been presented with the unprecedented opportunity to be extraordinary and make some leaps to improve the patient journey for millions of people around the globe? During the 24 hours of live and pre-recorded content we pressure tested some of the biggest trends facing the industry as companies of all sizes, in all sectors, grapple with the new reality and strive for the extraordinary.

As part of 13 amazing panels, which are available on-demand at pharmavoice100.com, we explored themes ranging from collaboration to diversity and inclusion to leading global teams to clinical trials to marketing practices to funding to the industry's reputation to the patient voice to the trusted relationship between sponsors and providers, which is essential as part of the drug development process. Over the course of the 24 hours, more than 60 of the industry's most inspiring leaders who are innovating, collaborating, and imagining what's next provided their expertise and insights. We can't thank them enough for their generous contributions and insights to make this event a great success. In addition to the panels, there is also engaging content that explores lifestyle journeys and polls to register your feedback on current issues facing the industry.

If we inspire you to raise the bar — in ways big or small — we would consider this a job well done.

Developing Clinical Trial Outsourcing Strategies that Scale As Biotechs Do
PANELISTS:
Amir Malka, President & Co-Founder, Bioforum the Data Masters
Guy Goldberg, Chief Business Officer, RedHill Biopharma
Kanchan Relwani, VP and Head of Medical Affairs, Alkermes, PharmaVOICE 100 — 2020
Sponsored by: Bioforum the Data Masters

Leading Global Teams
PANELISTS:
Adlai Goldberg, Global Digital, Social and Commercial Innovation Life Sciences Leader, EY, PharmaVOICE 100 — 2020
Guadalupe Hayes-Mota, Director, Global Supply Chain and Manufacturing, Ultragenyx Pharmaceuticals, PharmaVOICE 100 — 2020
Susanne Munksted, Chief Precision Officer, Diaceutics
Rebecca Vermeulen, VP Strategy Lead Patients and Society, Roche, PharmaVOICE 100 — 2020

Brand Stewards: Creating an Omnichannel Experience for HCPs and Patients
PANELISTS:

Dave Ormesher, Founder, closerlook, Red Jacket, PharmaVOICE 100 — 2013, 2011, 2010
Ryan Billings, Head, US Oncology Digital Marketing & Customer Experience, GSK, PharmaVOICE 100 — 2020
Mario Nacinovich, VP, Marketing, Eyevance Pharmaceuticals, PharmaVOICE 100 — 2011, 2005
Sponsored by: closerlook

Remote Clinical Trials: Advantages and Challenges
PANELISTS:
Bill Byrom, Ph.D., VP, Product Strategy and Innovation, Signant Health, PharmaVOICE 100 — 2017, 2008
Joe Dustin, Head of Clinical Innovation, Bristol Myers Squibb
Craig Lipset, Founder, Clinical Innovation Partners, Red Jacket, PharmaVOICE 100 — 2016, 2010
Sponsored by: Signant Health

Collaboration Across the Industry
PANELISTS:
Steve Matas, Senior VP, Strategic Resourcing, Advanced Clinical, PharmaVOICE 100 — 2020
Peter Anastasiou, Executive VP and Head of North America, ▶

PharmaVOICE 100 24-Hour Celebration Marathon

Lundbeck, PharmaVOICE 100 — 2020

Craig Serra, Global Head, Innovation Data Operations, Novartis, PharmaVOICE 100 — 2020 and 2017

Sponsored by: Advanced Clinical

Driving Commercial Relevance
PANELISTS:

Anshal Purohit, President, Purohit Navigation

Shannon Dahl, Ph.D., Chief Scientific Officer, Cell Care Therapeutics, PharmaVOICE 100 — 2018

Mary Frances Harmon, Senior VP, Corporate and Patient Relations, PTC Therapeutics, PharmaVOICE 100 — 2020

Kinnari Patel, Pharm.D., Chief Operating Officer & Head of Development, Executive VP, Rocket Pharmaceuticals, PharmaVOICE 100 — 2018

Sponsored by: Purohit Navigation

Unprecedented Public Support for Pharma: An Opportunity to Lead Like Never Before
PANELISTS:

Wendy Lund, CEO, GCI Health, PharmaVOICE 100 — 2020

Rob Jekielek, Managing Director, Harris Poll

Laura Randa, VP Market Access, HEOR and Public Policy, Mycovia Pharmaceuticals, PharmaVOICE 100 — 2018, 2013

Sponsored by: GCI Health

Diversity in Clinical Trials
PANELISTS:

Tami Klerr, Executive VP and Chief Business Development & Marketing Officer, PRA Health Sciences, PharmaVOICE 100 — 2012

Kent Thoelke, Executive VP and Chief Scientific Officer, PRA Health Sciences, PharmaVOICE 100 — 2013

Elizabeth Garner, M.D., Chief Medical Officer, ObsEva, PharmaVOICE 100 — 2019

Jessica Scott, M.D., J.D., Head of R&D Patient Engagement Office, Takeda Pharmaceutical Company, PharmaVOICE 100 — 2020

Sponsored by: PRA Health Sciences

Aligning Equity, Diversity, and Inclusion (EDI) with a Corporate Mission/Purpose
PANELISTS:

Eric Dube, Ph.D., President and CEO, Retrophin, PharmaVOICE 100 — 2020

Laurie Cooke, President and CEO, Healthcare Businesswomen's Association, PharmaVOICE 100 — 2017, 2009

Robin Shapiro, CEO, TBWA\WorldHealth, PharmaVOICE 100 — 2020

Sponsored by: Retrophin

Will Science Ever Marry Technology?
PANELISTS:

Suresh Kaata, Founder and CEO, Saama Technologies, PharmaVOICE 100 — 2018

Sagar Anisingaraju, Chief Strategy Officer, Saama Technologies, PharmaVOICE 100 — 2020

Elliot Barnathan, M.D., FACC, FAHA, Senior Director, Cardiovascular Clinical Development, Janssen Research & Development, PharmaVOICE 100 — 2020

Jay Kaminski, Advisor, Health2047 Inc.

Clive Meanwell, M.D., Ph.D., Founder, The Medicines Company, PharmaVOICE 100 — 2016

Sponsored by: Saama Technologies

Managing a Remote Workforce: Case Studies in Maintaining Business Continuity
PANELISTS:

Deb Tatton, Senior VP, Global Clinical Operations, Parexel, PharmaVOICE 100 — 2020

Erin Horvath, President, Distribution Services, AmerisourceBergen Corp., PharmaVOICE 100 — 2020

Nandini Ramani, Chief Operating Officer, Outcome Health, PharmaVOICE 100 — 2020

Jeffrey W. Sherman, M.D., FACP, Executive VP and Chief Medical Officer, Horizon Therapeutics, PharmaVOICE 100 — 2020

Sponsored by: Parexel

The Patient Voice
PANELISTS:

Barry Greene, President, Alnylam Pharmaceuticals, Red Jacket, PharmaVOICE 100 — 2019, 2018

Jack Barrette, CEO, WEGO Health, PharmaVOICE 100 — 2019, 2006, 2005

Barby Ingle, President, International Pain Foundation, PharmaVOICE 100 — 2020

Roslyn Schneider, M.D., Founder, RozMD Patient Affairs Consulting LLC, PharmaVOICE 100 — 2018

Sponsored by: Alnylam Pharmaceuticals

PharmaVOICE 100 24-Hour Celebration Marathon

What's Next ... Red Jacket Super Panel
PANELISTS*:

Al Altomari, CEO, Agile Therapeutics

Jeffrey Berkowitz, CEO and Director, Real Endpoints

Sharon Callahan, CEO, CDM & Chief Client Officer, Omnicom

Dr. Donald Deieso, Executive Chairman & CEO, WCG

Dr. Nancy Dreyer, Chief Scientific Officer & Senior VP, Real World Solutions, IQVIA

Dr. Cameron Durrant, Chairman & CEO, Humanigen

Barry Greene, President, Alnylam Pharmaceuticals

Dr. Amir Kalali, Chief Curator and Chairman, CNS Summit

Michele Keefe, President, Commercial Solutions, Syneos Health

Craig Lipset, Founder, Clinical Innovation Partners

Andrea McGonigle, Managing Director, Health & Life Science Business Development, Microsoft

Dave Ormesher, CEO and Founder, closerlook Inc.

Dr. Ahnal Purohit, Founder and CEO, Purohit Navigation

Mike Rea, CEO and Co-Founder, IDEA PHARMA

Julie Ross, President, Advanced Clinical

Leerom Segal, Co-Founder and Chairman, Klick Health

Wendy White, Co-Founder, RareiTi

*All panelists are PharmaVOICE 100 Red Jacket Honorees

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Lifestyle Journeys
Vipassana Meditation
Sponsored by: Cognizant

The Magical Mila Foundation
Sponsored by: Levlane

Polls
COVID-19 Experience
Sponsored by: Alkermes

Patient Behavior
Sponsored by: Health Union

Patient Recruitment
Sponsored by: KnowRare

Pharma's Reputation
Sponsored by: Ivy Cohen Corporate Communications

Real World Evidence
Sponsored by: RareiTi

Also, check out the Red Carpet Celebration video and the more than 80 videos featuring
PharmaVOICE 100 honorees throughout the years at www.pharmavoice100.com.

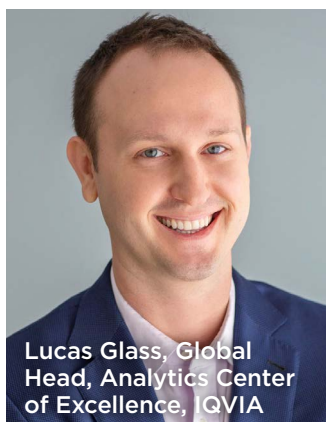**None of this could be possible without our sponsors, who also took the bold leap to join us on this odyssey. Thank you to:**
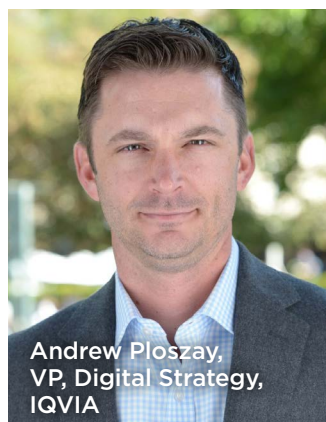
AI With a Healthcare IQ

A Conversation with Lucas Glass and Andrew Ploszay

Lucas Glass, Global Head, Analytics Center of Excellence, IQVIA

Andrew Ploszay, VP, Digital Strategy, IQVIA

Since the term was first coined in 1955, "artificial intelligence" (AI) has led to significant advances across industries. From robotics to automobiles, to digital personal assistants, and even online shopping. In healthcare, AI has enabled many successes, but a few noteworthy failures have made some companies question how they should apply AI to their clinical or commercial solutions.

Whether we're ready or not, AI is already here. According to some reports, more than 50% of executives expect broad scale AI adoption by 2025. A report from Global Market Insights forecasts that the U.S. healthcare AI market alone will exceed $10 billion by 2024. The global AI market has been growing rapidly as major pharmaceutical and medical technology companies begin investing heavily in internal AI efforts and external partnerships with data and technology companies.

Alongside this emerging technology, IQVIA recognizes its customers are facing an increasing need to compete and do everything from get drugs to market faster, predict outcomes with greater precision, turn unstructured data into insights, and scale to solve increasingly complex problems. Meanwhile, customers are finding that legacy approaches don't consistently meet these growing needs, but some are still hesitant to fully embrace AI.

To help, IQVIA dynamically integrates data, technology, advanced analytics, and the right expertise with AI capabilities to ensure that its application to any healthcare problem not only starts with the right ingredients but is also guided by the right experts. This dynamic integration happens thanks to the IQVIA CORE, which powers the company's AI and enables healthcare-grade solutions for customers.

Recommending a product that someone might want to buy in an online store is one thing — the cost of getting that wrong is likely minimal to the customer. When predicting the most likely diagnosis or treatment for a patient — getting it right is vital. IQVIA believes the key to success in healthcare is to apply "AI with a healthcare IQ," which means AI and machine learning capabilities developed, guided, and interpreted by not only data science experts, but healthcare experts as well.

Benefits to Clinical and Commercial Applications

Two such experts at IQVIA are Lucas Glass, Global Head, Analytics Center of Excellence, and Andrew "AJ" Ploszay, VP, Digital Strategy. On the clinical side, Lucas's team supports strategic decisions across clinical development for trial design, planning and execution. On the commercial side, AJ's team leverages technology to help pharma companies identify and reach the HCPs and patients that their therapies are designed to. Together, they have a broad and deep perspective on AI, and how it can help healthcare companies that embrace the technology today.

"There is a wide range of applications for AI capabilities across the clinical to commercial journey – it can get very complex – so it's important to start by articulating the benefits of AI in a very simple way," AJ says. "No matter where or how we apply AI to healthcare, the goal is to increase the probability that we get more precise answers, enhance speed, and be as agile or scalable as possible in a competitive landscape."

According to Lucas, "the stakes for clinical and commercial are very different, but accuracy, moving quickly, and being able to adapt to change is critical to both," he says. "In clinical, for example, we might be working to shorten the drug development cycle from 12 to 9 years. On the commercial side, we may be trying to optimize ROI or support compliance. In either case, the outcomes not only have the potential to benefit customers, but they'll also have an impact on patients as well."

AI in Clinical Development

Currently, AJ says there is considerably more AI activity on the clinical side compared with commercial applications, in part because clinically relevant data is more readily or publicly available. "As access to data from publications, patents for drug discovery, FDA submissions for competitive intelligence, and electronic health record (EHR) data becomes more readily available, AI can be used to curate information to drive better outcomes for commercial applications," he says.

According to Lucas, AI is instrumental in helping to drive clinical development forward. One primary example has been in the area of site selection. "Recommending sites for a clinical trial is a very data-driven aspect of clinical trials," he says. "Traditional approaches to site selection are based on existing partnerships or relationships. This doesn't consistently lead to the best performing sites. AI is changing that. More broadly speaking, some other valuable areas of application so far have been reduced administrative burden, increased predictability of portfolios, and enhanced trial recruitment." In working with customers, for example, IQVIA has seen up to 46% faster site identification and 30% faster clinical trial recruitment on average when leveraging AI capabilities compared with legacy approaches.

Despite these benefits, there is still hesitance in some areas to fully adopt AI capabilities. According to Lucas, one of the primary areas of concern in terms of adoption is a disconnect between the data scientists and the decision makers in the field. "To over-

come this obstacle, we are making massive investments in market-facing technology in collaboration with our subject matter experts (SMEs)," he says. "We have a decision intelligence team that focuses on the barriers to trust and adoption of AI such as psychology, UX, and decision theory. We have integrated the technology and operations teams so that they are deeply engaged in the agile process of development. We are building a network of hospitals and vendors that can make the necessary data available, in private, secure ways to deploy technology into the health systems more seamlessly."

By adopting these strategies, Lucas hopes that his team can further reduce some of the other barriers of adoption, such as the complexities around the federated universe of Persona Health Information (PHI) clinical data; the high level of expertise needed to run a clinical trial, which leads to adoption challenges; the highly specific processes across the industry that lead to a challenging landscape for tech investment; and less mature level of clinical technology versus other industries, such as financial technology and marketing — all of which lead to difficulty with deployment of AI that is required to achieve value.

"If the algorithms that recommend clinical trial strategies can account for market dynamics, organizations can make more informed decisions," he says. "For example, the recommended strategy for a trial that is racing against a competitor should be different than one in a less competitive environment. It may be worthwhile to create a much more aggressive albeit expensive trial strategy."

Lucas says key factors for sponsors, sites, and clinical teams to consider when looking to integrate AI into their processes is to focus on the end use and the user experience; ensure that the applications leveraging AI are advanced enough for the technology; and engage SMEs early and often with the AI that you are intending to build — and share your solutions to their problems regularly.

Even with all these applications to clinical development, Lucas believes we're only just beginning to scratch the surface. "The integration with clinical research as a care option is the most profound value that AI can provide," he says. "Imagine a world where physicians can personalize medicine to such an extent that the treatment they prescribe can be either a marketed product or one in development. The clinical development landscape is too dynamic for physicians to bridge that clinical and commercial gap on their own. With the support of AI, that becomes possible."

AI in Commercialization

The use of AI in commercial applications can, as Lucas explains, be used to build a connection between clinical and commercial operations, as well as transform the sales and marketing capabilities of pharma organizations by identifying patient pathways, improving disease detection, enhancing multi-channel marketing, heightening smart targeting, pinpointing next best actions, optimizing field force activities, and improving forecasting.

"Commercial models have radically changed in the last 10 years," AJ says. "Pharma companies have had to reinvent the way they engage with healthcare providers (HCPs). One-on-one meetings, team-to-team engagement, and opportunities to gain access to HCPs are dwindling."

One of the primary reasons legacy approaches to HCP engagement aren't achieving results is because physicians are inundated with information and promotions across so many channels, which becomes noise that's easily ignored. It's difficult for them to discern what's relevant or not, and it's even harder for pharma companies to make these once valuable connections.

"AI is essential in helping to optimize multi-channel engagements by supporting the delivery of the right message on the right channel to the right customer," AJ says. "This requires the orchestration of different types of engagements with HCPs, so that marketing and sales operations plan and execute well-coordinated personal and digital interactions that take into account each HCP's preferences. Ultimately, AI then brings together all this engagement data for fast-paced, informed decision-making. When used well, AI leads to a more relevant and impactful customer experience." In one example, IQVIA helped a customer achieve 85% accuracy in HCP targeting, while another achieved a 33% greater speed to insight by optimizing a multi-channel marketing approach with AI.

"Another way it can be used effectively in commercial problem-solving is in the form of augmented intelligence," AJ continues. "Traditionally, when we think of artificial intelligence, we might think of how our phone can learn our daily routine and make recommendations about the fastest route to commute to work. From the user perspective, this all happens behind the scenes and feels very automatic."

With augmented intelligence, AI is used as an aid to human judgment to generate decision-ready data to assist human intelligence and decision-making. "With this approach, we're starting to see companies use AI to make more informed decisions around areas such as resource allocation, whether that's financial or human," AJ says. "For example, AI can leverage real-time HCP data to provide recommendations to sales teams for how best to prioritize weekly customer activity. It then informs the optimal sequence of engagements, digital or personal, to engage in the following months. It also provides recommendations for future budget allocations across channels based on calculated ROI data of past investments."

While we're already seeing promising examples like these in practice, AJ says the application of AI for the commercial space is still in its infancy and facing two major barriers: establishing causation in an "influence-oriented" situation, and the scarcity of available performance data in some countries. In this regard, a better connection between clinical and commercial operations will be a game changer, helping to boost the next phase of AI development in pharma companies.

AJ says the companies that are using AI with success, including IQVIA, are carefully experimenting on a grand scale. "It's a phenomenally exciting time to be in healthcare and the life sciences, maybe the best time ever," AJ says. "At the same time, incorporating AI and machine learning into various processes is like trying to play chess on multiple boards that are all moving at different times. It's important that we explore new areas, but we also have the get it right. That's where the 'healthcare IQ' comes into play. We're applying AI to help our customers, but we never lose sight of the fact that, ultimately, everything we do for customers is ultimately for the benefit of patients."

Lucas and AJ both agree that the potential benefits of AI to the healthcare and life-sciences industries — from drug innovation through to commercialization — are undeniable. Even though it's widely applied across industries, AI can still feel like an emerging technology.

"AI is already becoming a prerequisite to success," AJ says. "I believe that pharma and other healthcare companies that adopt and internalize it, with the right approach and expertise, will be best positioned to develop treatments or even cures faster, drive greater efficiencies, and stay ahead of the competition. And when we talk about achievements like early diagnosis, or recommending the right treatments, that means AI can be life-changing for patients, too." ∎

This article was created for and commissioned by IQVIA.