# Global Life Sciences Summit: Future of Life Sciences

May 17, 2023

From a “one-size-fits-all” approach to mapping “the right drug for the right patient,”—precision medicine has led to a paradigm shift in healthcare. This offers huge opportunities and challenges for diagnostic and life science companies, who must act strategically to optimize patient journeys and synergize across the ecosystem to ensure meeting future market needs. As they navigate the next frontier of personalized treatments, therapeutic innovation, continuous learning, and patient empowerment will play key roles in improving health outcomes and building trust.

In the last session of the summit, panelists discussed the future state of life sciences—advancements and challenges in implementing precision medicine trials and the evolving journey of patients in neurodegenerative diseases (NDDs) treatments.

## Roadblocks on the path to precision medicine

Rapid technological advancements such as next-generation genome sequencing and molecular-targeted drug delivery have presented a new paradigm in therapeutics—precision medicine. The underlying concept of precision medicine is to delimit diseases using patient data, from digital health metrics to genetics, that facilitates a “personalized” yet evidence-based treatment.

The first precision medicine came around 25 years ago with Herceptin's launch in the market. By focusing on the molecular characteristics of the patient, rather than the conventional one-size-fits-all approach, therapeutics can become more patient-centered.

Alasdair Milton, Managing Director in KPMG Healthcare and Life Sciences, shared how healthcare has made remarkable strides in treating chronic diseases like cancer through diagnostic testing to match the right patient to the right therapy. Although a new era of medicine is revolutionizing personalized care, “a recent JCO precision oncology report indicated diagnostic testing-informed treatments benefitting only one-third of advanced non-small cell lung cancer patients with potentially actionable biomarkers,” he highlighted.

In the first segment, Luca Dezzani, Vice President of US Oncology Medical Affairs at Johnson and Johnson (J&J), along with Terri Conneran, Founder of KRAS Kickers and a lung cancer survivor, shared their insights on the roadblocks preventing precision medicine from fulfilling its potential.

The effective use of biomarker testing plays a fundamental role in identifying the best treatment options to transform patient outcomes. Terri spoke about the lack of information accessibility to different biomarkers affecting the lives of many. “It took me three years and a second opinion to find out that I had a biomarker called KRAS,” she said. This is why she founded the KRAS Kickers community—a tumor-agnostic international biomarker group—to educate and engage patients with the KRAS biomarker. She believes patients should be empowered with accurate information to actively participate in their health care decisions.

Even with targeted therapy, precision drugs are not without side effects. Speaking of the preciseness of precision medicine, Terri stated, “It boils down to having comprehensive diagnostics before you get the first treatment. You need to be so strategic coming into treatments up front. We need effective diagnostics so that it leads to the best treatments.”

Luca stressed a targeted therapy does not mean no side-effect. “Every target is pretty much a story, a new journey in itself,” he said. If we consider KRAS, the understanding of its mutations is constantly being updated. It has moved from being an undruggable target to druggable in cancer. This makes the ecosystem of precision medicine quite complex.

He also discussed how pharmaceutical and healthcare companies historically have been healthcare professionals (HCP) centered. Now patient-centered care is the way of doing things. Data sharing and partnership across the different players within the healthcare ecosystem are critical to enable continuous learning as we innovate and create precise drugs that can truly hit a specific biological or molecular target.

Terri backed this by saying, “Patients need to be included in every part of this equation.” From identifying protocols and leveraging research to better understanding the gaps—including patients in every aspect of their precision medicine journey can make a positive difference. Recognizing different demographics culturally, helping them understand genetic mutations, and giving them access to the knowledge for best treatment opportunities can truly empower patients and their loved ones to fight through chronic diseases. “This is not a matter of top-down learning. This is a matter of relationships. This is a matter of side-by-side learning with patients, bringing in different stakeholders, researchers, oncologists, and patient care partners. That's our empowerment,” she concluded.

Luca further discusses how patients can be empowered to ask for biomarker testing as it's imperative to enable their precision medicine journey. Science is evolving fast, due to which physicians are struggling to keep pace with genetic abnormalities and their targeted drugs. “I think the idea that we still need to ensure appropriate education for HCPs is critical,” he shared. On the other hand, patients can help accelerate the process by taking an active role in their personalized treatment journey, by sharing their stories within the community.

Drawing attention to the payers in precision medicine, Alasdair spoke about the complex and fragmented structure of the U.S. payer landscape. Pharma must engage with healthcare payers and address the total economic impact of new drugs from their perspective. Because genetically targeted therapies can only be widely accessible if payers opt to cover them, Terri and Luca both alluded that collaboration among payers, HCPs, and clinicians is essential for fast-tracking the uptake of precision drugs and value creation.

## The evolving journey of patients in NDD treatments

The final segment of the summit featured Varun Renjen, Managing Director at KPMG Healthcare and Life sciences Advisory practice; Dr. Howard Fillit, Co-founder and Chief Science Officer for the Alzheimer's Drug Discovery Foundation; and Pearl Pugh, President of J&J Neuroscience. Together, they walked everyone through the future state of personalized medicine in NDDs.

We are on the precipice of precision medicine in NDDs. Historically, NDDs have been thought of as neurological diseases, but thinking needs to evolve to capture its systemic nature and drive future treatments. Varun highlighted some of the recent developments in the precision medicine and NDDs space including identification of alpha-synuclein as the first Parkinson’s disease gene along with the FDA approval of a new ALS drug. “With new treatments and new biomarkers, we’re able to truly harness the power of precision medicine in NDDs, which we've seen in oncology,” he said. This can further help optimize the patient journey through timely diagnosis of diseases, matching the right patient to the treatment, and monitoring the progress.

Speaking of the biggest unmet needs in NDDs, Dr. Fillit stressed on early diagnosis and test as a health imperative. He stated, “I think as a part of the precision medicine, these drug approvals are really enabled by biomarkers, particularly by PET amyloid scans that are not covered by insurance presently.” This can change going forward and patients might be able to get an accurate diagnosis based on these scans or blood tests and get treated to remove the amyloid and slow the rate of decline.

Pearl added to it by emphasizing increased patient and caregiver education pertaining to NDD signs and symptoms. While access to care remains a challenge for many patients, particularly those living in rural communities, healthcare providers need to meet patients where they are—to educate them and prioritize improving access through innovative delivery models.

Dr. Fillit further shared his insights into the importance of early screening for NDD patients. Research shows that the average time from noticing the first symptoms by a loved one to an actual diagnosis from the doctor is about two years. “And the problem with that is in those two years adverse events can occur, for example, unnecessary hospitalizations that are potentially avoidable because many elderly people have comorbidities like diabetes and they're not taking their

medication properly because they have cognitive impairment," he alluded. Stigma and nihilism around the diagnosis of NDDs like Alzheimer's act as barriers to early diagnosis, which healthcare providers need to actively address and dispel.

A positive diagnosis usually requires doctors to spend time with the patients and their families, explaining to them the challenges of NDD, expected day-to-day caregiving, and the treatment opportunities. In the U.S. physicians spend about eight to eleven minutes on average for each patient visit. "Studies show even doctors get reluctant to make an early diagnosis because it requires education and counseling, which takes up time that they're often not paid for," he added.

Pearl highlighted the pandemic has further worsened the scenario. Many patients have missed their regular screenings during the pandemic and are still hesitant to visit primary care physicians to discuss their comorbidities. "The increased burnout and turnover across the healthcare provider community can also result in delays for patients being diagnosed by specialists," she stated. Creating awareness and educating patients and caregivers to seek medical consultations for early symptoms is crucial.

The biggest challenge in NDDs like dementia is patients have pathological changes years before they have any symptoms. Treatments are more likely to work better if the disease is intercepted early. One of the unique features of Alzheimer's is that people eventually lose cognitive function, their ability to function on a day-to-day basis. This is why caregiver education and training is imperative to provide quality, tailored care to NDD patients.

From a patient-caregiving dyad perspective, Pearl further underscored the importance of open and well-informed communication. Creating opportunities to learn about the disease can help reduce stress and strengthen the process of caregiving. "The more we educate patients around disease awareness, provide access to tools and resources, they can feel empowered to advocate for themselves and really have a good dialogue, as counterparts of the health care provider community," she suggested.

Drawing attention to the success of precision medicine in oncology, Pearl highlighted how scientific innovations have improved drug targeting significantly over the past years. However, in neuroscience, a globally accelerating aging population and the rising prevalence of NDDs made it difficult to apply precision medicine historically. Today greater disease understanding, innovative diagnostic approaches, and digital biomarkers are enabling early detection and monitoring of targeted NDD treatments. One of the key barriers here is the shortage of counselors in the healthcare space to educate patients about genetic and familial risk. Ensuring payer coverage is also a critical factor in advancing the adoption of complex, personalized medicine therapies within the community.

Dr. Fillit further discussed personalizing precision medicine with combination therapies. "We're already giving a 'standard of care' that has a combination of drugs based on precision medicine with novel biomarkers. People can get, say, cholinesterase inhibitors and a monoclonal antibody infusion to remove amyloid along with a neuro anti-inflammatory drug that recalibrates epigenetic regulation of aging or mitochondrial dysfunction in Alzheimer's," he alluded.

From the view of patient's journey, it's important to consider the risk of developing dementia at older ages. These patients are elderly, frail, and often decline in their cognitive abilities in about one year or sometimes six months if they're not treated. Although the monoclonal drugs help slow the rate of decline by 25-30 percent, intaking these antibodies are burdensome. Dr. Fillit is hopeful that with evolved combination treatments, precision medicine can ease the burden of taking these drugs for the patients and their loved ones, either through subcutaneous administration or oral bioavailability.

Varun also pointed to the need for maximizing market access, particularly as therapeutics get more complex, to ensure patients can access personalized medicine treatments to improve their health outcomes. Infusion centers, like in Manhattan, New York, have the ability to offer treatments that are tailored to each patient's needs. "These independent centers are infusing drugs not just for cancer but for multiple sclerosis and other diseases like rheumatoid arthritis," Dr. Fillit suggested.

The summit wrapped up by stressing the need for policymakers, payers, HCPs, and the patient community to work collectively to bring attention to the current challenges and significant unmet needs in precision medicine. "We know there are many advocacy groups who can help educate policymakers and payers about those needs and what they experience. Our responsibility as a leader, here at Janssen, is to really bring in some of these insights to help inform policies to address the growing affordability gap, foster a patient-centric healthcare system, and enable our unique ecosystem of innovation. We want to create a future where serious mental disease, neurodegenerative diseases are a thing of the past," Pearl concluded.