Parkinson’s Pipeline: Promise in Research and Treatment

At the Michael J. Fox Foundation’s Parkinson’s Disease Therapeutics Conference, experts revealed encouraging new directions in treating and understanding PD.

By Ted Pigeon, Editor-in-Chief

The Michael J. Fox Foundation held its annual Parkinson’s Disease Therapeutics Conference in New York in November, bringing experts together from around the world for an educational forum about the latest innovations in the field. Event chair Darryle Schoepp, PhD, Vice President and Therapeutic Area Head of Neuroscience at Merck, noted that research and development in Parkinson’s disease (PD) is now rapidly gaining momentum. “In the past it was hard to get traction, but the science has advanced tremendously.” Now, Dr. Schoepp said, “Many more companies are working in Parkinson’s disease.”

Presentations throughout the day highlighted several avenues that may lead to enhanced understanding of the disease itself as well as the development of targeted therapeutic approaches. For instance, Lars Wahlberg, MD, PhD, President and CEO of NsGene, Inc., described an investigational therapy called encapsulated cell therapy, which enables the extended application of Glial Cell Line-Derived Neurotrophic Factor (GDNF), a survival and axonal regenerative protein. He observed that the modality represents “an innovative platform suitable for multiple biologics, is implantable, retrievable, and replaceable.” Noting that Phase 1b trials are currently underway, Dr. Wahlberg, concluded that encapsulated cell therapy, “has matured from an experimental device to clinically applicable platform.”

Other potential therapies include leucine-rich repeat kinase 2 (LRRK2) inhibitors. Marco Baptista, PhD, Senior Associate Director of Research Programs at the Michael J. Fox Foundation, pointed out the foundation has established collaboration between major drug manufacturers—including Genentech, Pfizer, and Merck—to address key questions about safety of LRRK2 inhibitors. Studies are ongoing, but the current data suggest that LRRK2 kinase inhibitors may slow the degenerative process and may play a pivotal role in the future.

In the realm of symptomatic therapies, Kathleen Poston, MD, MS, Assistant Professor of Neurology and Neurological Sciences at Stanford University, discussed the potential for MRI biomarkers to help predict cognitive impairment in PD. “Cognitive impairment has in many ways been ignored,” Dr. Poston offered. “When someone is motorically disabled, we are not worried about whether their memory is working well, nor can we test it effectively.” Dr. Poston suggested that connectivity changes in default-mode network regions could be due to concomitant AD-type pathology in PD.

Another focal point of the conference was the Parkinson’s Progression Markers Initiative (PPMI). Multiple speakers examined the latest developments related to PPMI, notably Andrew Singleton, PhD, Senior Investigator for the Laboratory of Neurogenetics at the NIH. Dr. Singleton described how he and his team used the PPMI to build a predictive model for early and accurate diagnosis of PD. He concluded that epigenetics could play an important role in continued efforts to reveal clinically and etiologically distinct forms of PD.

Among other topics explored, C. Warren Olanow, MD, Professor of Neurology and Neuroscience at Mount Sinai Hospital in New York, addressed the impact of levodopa delivery approvals on the future of dyskinesia therapeutics. Citing continued advances in research and refinement of therapies, Dr. Olanow stated that levodopa delivery systems are moving toward being able to provide continuous deliverability using methods that are less invasive. “The hope is that one day we can provide treatment with all the benefits of levodopa with no motor complications and no side effects,” he noted.

Despite the promise for development of novel approaches for treating PD, clear challenges remain. Costs continue to soar, said Ray Dorsey, MD, MBA, Professor of Neurology at the University of Rochester. These trends are in large part due to the lack of sensitive objective measures, he said. However, ingenuity both within and outside the pharmacologic scope may accelerate therapeutic development. One tool that may be useful is the smart phone. Dr. Dorsey suggested that data captured from smart phones can help differentiate those with PD from those without, as well as predict disease severity and detect pharmacological response to treatment. “Mobile technology has the potential to improve self-management, care, and research,” he said.