Apple Watch Parkinson Disease Symptom Monitor Is Cleared

A program that uses motion sensors built into the Apple Watch to monitor symptoms of Parkinson disease gained FDA clearance.

In addition to recording tremors and uncontrolled body movements, or dyskinesia, the StrivePD software from Rune Labs tracks medication use, adverse events, and patient-reported outcomes such as mood and overall health. When integrated with the Medtronic Percept PC Deep Brain Stimulator, it may also gather detailed brain activity information, a company statement noted.

The software device enables continuous monitoring of typically fluctuating signs and symptoms of Parkinson disease. This creates a comprehensive view of disease progression that isn’t possible with periodic in-clinic observations. Such data could be used to identify patients earlier in the course of their disease and tailor treatment more quickly, according to the statement.

“When people with Parkinson’s are prescribed new medications, adjusting how much to take and when to take it...can be a lengthy process. StrivePD helps people to track their symptoms and improvements, accelerating the time to an optimal medication schedule,” Aura Oslapas, who created the StrivePD app based on her own experience with Parkinson disease, said in the statement. Oslapas serves as a member of Rune Labs patient advisory board.

The software also could accelerate clinical trials by providing granular real-world data that would help select patients for studies, define trial end points, and accurately measure therapy impact, according to Rune Labs.

Action Plan Targets ALS and Other Neurodegenerative Diseases

In response to a law passed last year, the FDA has released a 5-year plan to develop safe and effective drugs for patients with amyotrophic lateral sclerosis (ALS) and other rare neurodegenerative diseases.

Known as the Accelerating Access to Critical Therapies for ALS Act, the law calls for creating an FDA task force and public-private partnerships in fiscal year 2022 as well as developing disease-specific science strategies over the next 5 years. These strategies will include improving understanding of disease pathogenesis by quantifying disease progression and identifying predictive biomarkers and by efficiently translating basic science to clinical studies. Clinical trial design will be optimized to enable early selection of promising therapeutic candidates and reduce drug development time and cost.

In addition, therapy developers will explore ways to facilitate patient access to new drugs by promoting greater clinical trial participation and reducing barriers for diverse populations. Digital health technologies and decentralized trial designs will also help, as will mechanisms for expanding access to novel therapies outside of clinical trials. “[P]atient engagement, public workshops, research projects, coordination across FDA centers and offices, and collaboration with the National Institutes of Health” will be key to the plan’s success, according to an FDA statement.

“We recognize the urgent need for new treatments that can both improve and extend the lives of people diagnosed with these diseases,” FDA Commissioner Robert M. Califf, MD, said in a statement. “[W]e need innovative approaches to better understand these diseases while also building on current scientific and research capabilities.” The action plan will enhance the quality of life for those with rare neurodegenerative diseases by facilitating access to new therapies, he added.

RNA Interference Therapy Approved for Hereditary Type of Amyloidosis

An RNA interference compound that halts or reverses polyneuropathy due to hereditary transthyretin (TTR)–mediated amyloidosis in adults has received FDA approval. Hereditary TTR-mediated amyloidosis is a rare, inherited, rapidly progressive, and fatal disease with debilitating polyneuropathy, for which few treatments are available.

A new molecular entity, vutrisiran is a chemically modified double-stranded small interfering RNA that targets variant and wild-type TTR messenger RNA in the liver. This reduces TTR serum levels and tissue deposits, which are thought to cause polyneuropathy. Marketed as Amvuttra, vutrisiran is injected subcutaneously once every 3 months, a more convenient option than its forerunner, patisiran, which is administered by infusion once every 3 weeks.

The FDA approval is based on a phase 3 clinical trial involving 164 patients—122 were randomly assigned to receive vutrisiran and 42 served as a reference group that took patisiran. In addition, 77 participants from the placebo group in a previous patisiran study served as an external control. After 9 months, quarterly injections of vutrisiran produced rapid, sustained reductions in serum TTR levels similar to those of patisiran.

Compared with placebo, vutrisiran treatment provided significant improvement on a neuropathy impairment scale: a mean 2.2-point reduction, or improvement, vs a mean 14.8-point increase, or worsening, of signs and symptoms. The vutrisiran group also experienced significant improvements in quality of life, nutritional status, and 10-m walk test results compared with placebo.

Common adverse events in the vutrisiran group were arthralgia, dyspnea, and a decrease in serum vitamin A levels. Patients who take vutrisiran should also take a vitamin A supplement at the recommended daily allowance, according to the drug’s label. – Howard D. Larkin

Note: Source references are available through embedded hyperlinks in the article text online.