FDA Approves Tolvaptan for ADPKD

Osaka Pharmaceutical’s drug Jynaqe (tolvaptan) received US Food and Drug Administration (FDA) approval as the first drug treatment in the United States to slow kidney function decline in adults at risk of rapidly progressing autosomal dominant polycystic kidney disease (ADPKD). The drug is a selective vasopressin V2-receptor antagonist. Tolvaptan showed a greater reduction in estimated glomerular filtration rate compared to placebo meeting the primary endpoint of the REPRISE trial (Replicating Evidence of Preserved Renal Function: an Investigation of Tolvaptan Safety and Efficacy in ADPKD).

The data appeared in a late-breaking abstract at ASN Kidney Week 2017 and were simultaneously published online in the *New England Journal of Medicine* (1).

Tolvaptan caused “more elevations in aminotransferase and bilirubin levels,” according to the *NEJM*. “The efficacy and safety of tolvaptan in patients with later-stage ADPKD are unknown.” After tolvaptan therapy was ended, the results were reversible for aminotransferase, and bilirubin levels did not exceed twice the upper limit of the normal bilirubin range, the *NEJM* noted.

“We are thrilled to be a part of this first milestone in treatment for ADPKD,” said PKD Foundation CEO Andy Betts. “For the past 35 years, our goal has been to walk with PKD patients every step of the way. It is gratifying to play a part in the inception of the discovery of this treatment, and to see it come to fruition. We hope that this is just the beginning of a new chapter for adults at risk of rapidly progressing ADPKD who suffer from the disease.”

Jynaqe is available only through a restricted distribution program, according to FirstWord Pharma. Patients must have testing for blood alanine and aspartate aminotransferases levels, as well as bilirubin, before initiating treatment with tolvaptan. Patients also must be tested at two weeks and four weeks after treatment begins, as well as monthly for 18 months and every three months afterward.

The company noted that the drug will be sold in a 28-day treatment pack at a wholesale cost of $13,041.10.

**Reference**


Puerto Rican Patients, Saline Industry Still Recovering

In mid-April 2018, Puerto Rico again experienced a total blackout, this time because of an electrical subcontractor’s mistake, not an act of nature like Hurricane Maria, which devastated the island in September 2017.

Many patients have to travel far—sometimes up to 12 hours—to find functional dialysis opportunities. In March 2018, Puerto Rico's health secretary said the department was working to bring in mobile units for dialysis. Luis Emanuelli, regional vice president of Fresenius Kidney Care, said he is aware of plans for the mobile units but is unaware of the timeline for bringing them on line.

To assist in getting help to people affected by Hurricane Maria, the National Kidney Foundation (NKF) set up a special fund for those needing dialysis. ASN joined NKF as a sponsor in the effort to raise money. Other organizations, and dialysis manufacturers and providers, joined in the effort:

- Akemia Therapeutics, Inc.
- Alliant Health Solutions
- Amgen Foundation
- American Renal Associates
- American Medical Technologists
- American Society of Nephrology
- American Society of Pediatric Nephrology
- DaVita Healthcare Partners
- Dialysis Clinic, Inc.
- Fresenius Medical Care
- Keryx Biopharmaceuticals
- National Renal Administrators Association
- OPKO Renal
- Relypsa, Inc.
- U.S. Renal Care

The hurricane-induced problems are a two-way street of dependence. The United States is very low on saline bags for dialysis, many of which are manufactured in Puerto Rico. The *Washington Post* reported that supply issues were made worse by the impact of Hurricane Maria on the medical products manufacturing sector in Puerto Rico, which affected small volume IV bags.

A statement from the US Food and Drug Administration (FDA) noted an effort to preserve the current stock of resources provided by Puerto Rican manufacturers. The FDA urged companies to submit data to extend expiration dates on products that could be stored for longer periods without safety issues.