MedicalXpress

Newly-developed drug for rigid muscles moves ahead in clinical trials

September 24 2014

A new formulation for a University of Minnesota-developed drug targeted at rare disorders is under development, with the potential to help a small number of Americans each year. The new drug formulation is designed to prevent serious complications that can occur when drug delivery is interrupted.

Individuals experiencing dystonia (abnormal muscle tension) or spasticity (muscle stiffness or rigidity) caused by cerebral palsy, multiple sclerosis, brain or spinal cord injury are frequently managed with baclofen, a muscle relaxant drug. Baclofen is currently available as an oral tablet and as an intrathecal solution, the latter being administered via a medical device resembling an insulin pump found under the skin.

If illness or mechanical problems with the intrathecal pump occur or if a patient cannot take the drug by mouth, baclofen administration can be interrupted. This interruption may result in baclofen withdrawal syndrome, a serious complication. Withdrawal can result in increased stiffness, seizures or even liver and kidney failures.

The newly developed intravenous (IV) drug formulation has passed an initial phase I clinical trial and is now entering a second phase I dosing escalation trial to examine the effectiveness and any potential side effects of the drug in higher doses. This formulation is anticipated to more effectively manage, and even prevent baclofen withdrawal syndrome for many patients.

The University has partnered with Allaysis, LLC, a pharmaceutical company focused on bringing orphan drugs to market, located in Philadelphia, to advance the drug through clinical trials and on to anticipated sales and distribution.

The new route of administration is expected to benefit a small population of patients like those seen at Gillette Children's Specialty Healthcare in Minnesota by baclofen IV clinical research lead and University adjunct professor Robert Kriel, M.D. Each month, two to three patients develop baclofen withdrawal syndrome at the hospital. "Scheduling for surgery is pretty complicated right now for someone on oral baclofen," said Kriel. These patients are sometimes unable to take baclofen by mouth for safety reasons before surgery.

"This is a patient population that has very frequent surgical needs," said IV baclofen research lead James Cloyd III, Pharm.D., who directs the University's Center for Orphan Drug Research and is a professor in the University College of Pharmacy. "So we went back to the lab to develop a new tool to manage spasticity when surgery comes up. We asked: can we administer baclofen through an IV?"

The proposed research is designed to answer that question. As little as three years time to market could be possible if IV-delivered baclofen succeeds in future clinical trials. Food and Drug Administration-designated orphan drug status, if approved, will contribute to the accelerated development process. Rough estimates suggest fewer than 10,000 Americans may have a need for IV-administered baclofen each year.

"Allaysis is very excited to partner with the University of Minnesota to help bring this lifesaving product to patients in need. Allaysis was started with one goal in mind, to bring drugs with very small patient populations to market. By forming this strategic alliance with UMN, we get closer to

fulfilling our mission," said Adolfo Gomez, president and CEO of Allaysis, LLC.

Provided by University of Minnesota