Sickle Cell Drug Candidate Under Development

For the first time, a pharmaceutical company has acquired a drug candidate developed with resources from a National Institutes of Health (NIH) program for rare and neglected diseases (http://l1.usa.gov/1kDK7XW).

The drug, called Aes-103, treats sickle cell disease by binding directly to hemoglobin and changing its structure, thereby reducing the sickling of red blood cells. It is the first drug specifically developed to target the underlying molecular mechanism of sickle cell disease.

Despite promising data on Aes-103, AesRx, located in Newton, Massachusetts, had difficulty securing private financing. Researchers within NIH’s Therapeutics for Rare and Neglected Diseases program signed a collaborative agreement with AesRx in 2010 and established a project team made up of government and industry scientists. The Therapeutics for Rare and Neglected Diseases program exists within NIH’s National Center for Advancing Translational Sciences, which aims to bring together the necessary collaborators to overcome obstacles to translating basic research into clinical applications.

After preclinical studies led to an investigational new drug application and clearance with the US Food and Drug Administration, Aes-103 moved into phase 1 and 2 clinical trials. Baxter International recently acquired AesRx and now plans to advance the drug’s clinical development.

FDA Alert on Pure Caffeine Powder

The US Food and Drug Administration (FDA) has issued a warning to consumers about powdered pure caffeine, especially products that are sold in bulk bags over the Internet (http://l1.usa.gov/Ugb7Gn).

One teaspoon of pure caffeine is roughly equivalent to the amount of caffeine found in 25 cups of coffee, according to the FDA. Symptoms of caffeine overdose may include rapid or dangerously erratic heart beat, seizures, vomiting, diarrhea, stupor, and disorientation. The FDA is aware of at least 1 death of a teenager who used the products.

The FDA notes that it is nearly impossible to accurately measure powdered pure caffeine with common kitchen measuring tools, making it easy to consume a lethal amount. The agency is also concerned about the increasing amounts of caffeine in various food products and beverages, such as gum and energy drinks, that may be attractive and readily available to children and adolescents.

The FDA considers 400 mg of caffeine per day—approximately 4 or 5 cups of coffee—as an amount that is generally safe for adults. There is no set level of caffeine intake for children and adolescents, but the American Academy of Pediatrics discourages them from consuming caffeine and other stimulants.” —Tracy Hampton, PhD