Research highlights need to address hemophilia in developing world

November 16 2009

When modern medicine finds a way to treat a medical condition, people often think that the problem is solved. But we also have to find ways to get that treatment into the hands of those who need it. For example, new research from North Carolina State University shows that much more needs to be done to help get existing treatment to hemophilia patients in the developing world, and that the current lack of treatment there is costing lives.

"This research illustrates international disparities in treatment for a disease that we know how to address," says Dr. Jeff Stonebraker, an assistant professor of business management at NC State and lead author of two new studies on hemophilia prevalence and treatment. "What we've found highlights the work that needs to be done by governments, health officials and pharmaceutical manufacturers to address the needs of those suffering with hemophilia in the developing world."

Type A hemophilia is a hereditary bleeding disorder that affects about 400,000 people - predominantly men - around the world. The disorder prevents blood from clotting normally, creating the risk of serious bleeding or internal bleeding. Ultimately, the disorder can result in severe pain, joint deformities and death in childhood or young adulthood. But the condition can be treated by replacing the missing clotting factor VIII, which enables those suffering from hemophilia A to live relatively normal lives. Unfortunately, the treatment is expensive and new research shows that access to that treatment is severely limited.

"This is the best data there is on a relatively small global population," says Mark Skinner, president of the World Federation of Hemophilia. "It will be incredibly useful to our members, to manufacturers of treatment products, ministries of health and researchers. These two very important papers help us see where progress is being made and how we can learn lessons that will help us continue to improve care and work toward our goal of treatment for all."

In the first study, the researchers found that prevalence of hemophilia A in high-income countries was approximately 12.8 per 100,000 males. The prevalence in lower-income countries was approximately 6.6 per 100,000 males. "The medical community tells us that the incidence of hemophilia A - or the number of people born with the condition - is the same around the world," Stonebraker explains, "so the difference in prevalence - or the number of people living with the condition at any given moment - appears to be due to much higher mortality in developing countries."

The study also showed that prevalence of hemophilia has increased over the past 30 years, as treatments for the disorder have improved. In other words, better treatment is helping those with hemophilia A live longer. For example, the United Kingdom had a prevalence of 9.3 per 100,000 in 1974, but it had risen to 21.6 per 100,000 by 2006.

In a second study, Stonebraker and his colleagues found that decreased mortality related to hemophilia A is tied to a willingness - by government health-care agencies or private insurers - to pay for treatment. Correspondingly, the consumption of factor VIII drugs has increased significantly in developed countries - and that trend appears poised to continue, with high-income countries expected to consume more and more factor VIII drugs in the future.

Stonebraker says the two studies should be incredibly helpful as budget-

planning tools for insurance companies and those countries that provide national health care, as well as for pharmaceutical companies that will want to project the amount of factor VIII drugs they will need to make to meet market demand. But, Stonebraker says, "the studies also show how much more needs to be done to address hemophilia in the developing world."

More information: The first study, "A study of variations in the reported hemophilia A prevalence around the world," was co-authored by Stonebraker, Paula H.B. Bolton-Maggs of the Manchester Royal Infirmary, J. Michael Soucie of the U.S. Centers for Disease Control and Prevention, Irwin Walker of McMaster University and Mark Brooker of the World Federation of Hemophilia. The second study, "A study of reported factor VIII use around the world," was co-authored by Stonebraker, Brooker, Robert E. Amand of the Biotherapeutic Modeling Group, Inc., Albert Farrugia of the Plasma Protein Therapeutics Association and Alok Srivastava of Christian Medical College. Both studies will be published in a forthcoming issue of *Haemophilia*.

Source: North Carolina State University (news : web)