

To: Professor Norman C. Nevin
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Dear Professor Nevin,

The World Federation of Hemophilia is an international not-for-profit organization dedicated to improving the lives of people with hemophilia and related bleeding disorders. As we move toward our vision of achieving *Treatment for All*, we also look forward to the day that there will be a cure for hemophilia and other inherited bleeding disorders.

With proper treatment, people with hemophilia can live perfectly healthy lives. Without it, many will die young or, if they survive, suffer joint damage that leaves them with permanent disabilities. Tragically, only about 25 percent of the estimated 400,000 people with hemophilia receive adequate treatment. Achieving a cure will bring the opportunity of a better, safer, and longer life to countless thousands of patients around the world.

The development of gene therapy has great potential for a broad spectrum of hereditary and acquired diseases including hemophilia. Gene therapy's success in the pre-clinical hemophilia arena has been accruing for the past 15 years and proof of principle that gene therapy works in humans has been established in at least one hemophilia clinical trial. The World Federation of Hemophilia strongly supports building on these successes.

Through our various programs we are actively engaged in monitoring scientific and technological developments that can achieve successful cures or more efficacious treatment of inherited bleeding disorders. One of our roles is to foster debate and dialogue on this critical issue. We support clinicians and researchers working in the field to achieve this goal, and are most grateful for those patients who have participated in clinical trials.

Achieving a cure for hemophilia is a challenge that cannot be met by the efforts of one individual, organization, company, or country. A global approach and community effort is required. The best way to test the promise of gene therapy is through clinical trials that incorporate a commitment to patient protection and careful adherence to generally accepted guidelines for testing experimental therapies in humans. As new hemophilia trials are instituted, patient safety must remain a high priority, and institutional review boards must maintain rigor in approving informed consent procedures. These trials must also involve the active participation of physicians with long-standing experience in the clinical management of hemophilia.

We believe in the feasibility and promise of gene therapy. We are eager for this field of research to proceed. Much remains to be done to find a cure and for gene therapy to be a viable alternative for the treatment of inherited bleeding disorders. We are hopeful that gene therapy research advances expeditiously.

Respectfully,

A handwritten signature in black ink, appearing to read 'Mark W. Skinner'.

Mark W. Skinner
President, World Federation of Hemophilia

A handwritten signature in black ink, appearing to read 'David Lillicrap'.

Dr. David Lillicrap
Chair, World Federation of Hemophilia Gene Therapy and Novel Technologies Committee