

THE LATEST FROM THE GENE THERAPY

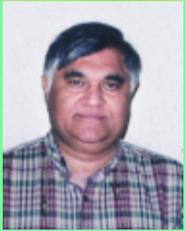
“Welcome to Camp La Jolla,”

proclaimed scientist Inder Verma in opening remarks at this year’s NHF gene therapy workshop hosted by Verma’s institution, the Salk Institute of Biological Studies, held from April 25 to 26, 2003 in La Jolla, California. It’s become a tradition at these meetings, which celebrate their 6th anniversary this year, to greet researchers, physicians and NHF staff as old friends and family. The workshops have recurred regularly due to the organizational efforts of a core group of scientists and NHF members—Inder Verma, Glenn Pierce, Michael Coyne, Katherine High, Steven Humes, Jude Samulski and Gilbert White.

BY JUDY BERLFEIN

Last year the conference was affectionately referred to as a family reunion. Just as family weathers hard times and celebrates good times, this group too has shared the highs and lows of gene transfer research. It is one step forward and a few steps back, commented more than one conference participant, reflecting on the state of hemophilia research.

Each workshop has been marked by a distinct emotion. In 1998 scientists were in awe over the rapid progress that researchers were achieving as hemophilic dogs showed signs of cure by gene transfer.¹ In 2000, the death of Jesse Gelsinger, the patient who died in a gene transfer trial from the metabolic disease ornithine transcarbamylase (OTC), a rare genetic deficit in the liver that renders it unable to clear ammonia from the bloodstream, dampened the excitement over newly-initiated clinical trials. The last two years seemed to bring renewed faith in gene transfer and a commitment to slow but steady advances with clin-



Inder Verma



Gilbert White



Glenn Pierce



Michael Coyne



Katherine High



Mark Skinner



Steven Humes

CONFERENCE

ical trials continuing to open. Now in 2003, with five phase I trials nearly completed, results have been mixed; the glass can easily be seen as half empty or half full depending on how the results are interpreted.

In addition, the field of gene transfer as a whole has taken a second blow. Two children treated with gene transfer for severe combined immunodeficiency have developed leukemia-like diseases with indications that gene transfer may be culpable.² Despite setbacks, scientists doggedly carry on, perhaps with a more realistic timeframe and an awareness of the need to build a strong foundation. Researchers seem to appreciate that getting the basics down will ultimately improve the safety and efficacy of any treatment.

Participants spent most of the workshop listening to short presentations on gene transfer research by experts in the field. During the final hour, a panel of individuals with hemophilia and parents

of boys with hemophilia presented their perspective on the state of research; all participants were invited to join in the discussion. The mood was one of tempered optimism. No one wanted to give up on gene transfer; however, panel members were frank about their concerns. Mark Skinner, who has moderate hemophilia, said his number one concern used to be

safety. He survived the blood disasters of the 1980s and feels confident treatment is now safe. These days, though, he worries about the cost. Will he be able to keep his insurance to pay for treatment? He wants to be sure researchers keep this angle in mind. A fancier, more expensive treatment won't solve the problem for the majority of those with hemophilia, he reminded scientists. That point was accentuated throughout the meeting as participants were reminded of the sobering facts: worldwide 400,000 people are affected by hemophilia. Less than one fourth receive regular treatment and only three percent are able to actually prevent bleeding episodes.

John Griffin of The Scripps Research Institute expanded on this sentiment. He challenged the community to widen its view and proposed that the goal should not be gene transfer per se,

but rather safe, effective and affordable treatment for all. Griffin continued: Why is the pharmaceutical community not developing second generation versions of factor, products with higher activity that stay in the blood stream longer, which could allow patients to treat once per week rather than two or three times a week? In response, Gilbert White of the University of North Carolina and member of the NHF's Medical and Scientific Advisory Council (MASAC) tried to convey the mindset of the pharmaceutical companies. Comparing gene transfer to the stock market and alternative therapies to bonds, he stated companies don't want to invest in bonds when the stock market is soaring. But now that the road to gene transfer appears less smooth, these companies may take a second look at alternatives. "I don't know if that's right or wrong," White commented, "but that is how it is."

The conference closed with reflections by the man who wears more hats than any single participant. Glenn Pierce has played a key role each year in organizing the gene therapy workshop. As a scientist with Avigen, Inc. and a person who lives with hemophilia, he comes to the field with a truly rounded perspective. "In some ways we may have missed the boat a bit," Pierce commented. "A couple of years ago, big barriers were coming up and we may have ignored them more than we should have. But it's not too late. We've cured thousands of mice and also several dogs for up to five years. We need to refocus and face these hurdles in a coordinated way. I hope in the next conference we'll have more answers than questions and that in this conference we've answered some of those key questions." 

SOURCES

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