# 2012 Symposium Highlights

The Fund held its 24th annual Scientific Symposium in Denver at the end of last month. One hundred and seventy-five people participated in the meeting, representing 15 countries. Dave Frohnmayer, Co-founder of the Fund and board advisor, said that the first full day of the Symposium was the "single most valuable day in translational medicine discussion that we've had since we formed the Fund." Special sessions on BMT and cell expansion techniques, together with sessions on drug and small molecules therapeutics, genome engineering and stem cells, and disease drivers made up the first day of three equally intense and productive days.

**Following are a few highlights from the Symposium as described by Scientific Advisory Board members Rich Gelinas, PhD, Grover Bagby, MD, Ray Monnat, MD and Jakub Tolar, MD, PhD:**

- Patient outcomes continue to improve as the major transplant centers pay ever closer attention to the best ways to both prepare patients for stem cell transplantation and to support them after a transplant.

- Representatives from four research teams reported that they are learning how to amplify or expand the number of stem cells in a sample of cord blood or peripheral blood. Two groups are already conducting clinical trials of an expanded cell treatment that is part of a regular stem cell transplant, but gives the recipient a burst of infection-fighting cells such as neutrophils much sooner than in previous protocols. If these stem cell expansion methods continue to improve, stem cell transplants should become safer, more effective and available for a larger number of patients.

- We are finally learning why bone marrow stem cells don't work well in people with Fanconi anemia. Studies with engineered FA mice now suggest that FA stem cells appear to be sensitive to DNA damage that arises from aldehydes that are naturally produced (and naturally removed) as cells grow. We still don't know which aldehyde in the cell is the most toxic, exactly how it affects DNA or hurts the stem cell. This breakthrough may lead to both the identification of toxic metabolites and new drug therapy based on speeding the removal of the toxic metabolite.

- Other studies in FA mice and with FA cells suggest that another cause of bone marrow failure may come from the tendency of certain FA cells to overproduce a chemical signal that usually leads to inflammation. This signal may suppress the growth of FA stem cells, or kill them. How this story connects with the aldehydes discoveries is unclear. This insight again has implications for drug therapies that could protect bone marrow stem cells.

The April 2013 issue of the FA Family Newsletter will include more details about the Symposium, including articles about key presentations.