

## Immunology today and new discoveries: building upon legacies of Dr. Robert A. Good

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### Robert A. Good Immunology Symposium



We know we are not yet at the finish line. But for us, the future is nothing short of miraculous and unimaginable.

Having mapped the entire human genome just a few years ago, investigators are now approaching their understanding of diseases on three separate levels. First, *Molecular Medicine*—medicine on the cellular level, giving us greater precision in identifying the genotype to treat the phenotype. Second, *Genetic Medicine*—medicine on the DNA level that allows physicians to offer patients more precise prediction. Finally, *Genomic Medicine*—the promise of treatment drawing upon the mapped human genome, by combining molecular and genetic research into medical practice.

Think of it! Today, from a single drop of blood, we can read the DNA, and look for which genes are present or missing, intact or broken, making specific diagnoses of disorders that only a short time ago were unheard of, uncertain, or misunderstood. From that,

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we can begin to decipher the consequences for a patient's life, health, and even longevity. Then move on to predictive, preventative, and personalized treatment, shaped and tailored to each patient's DNA—using massive information at our fingertips to materially impact on patient outcome. It has been characterized by some as *Information Based Medicine*—the right medication, for the right person, at the right time.

Add to this, the excitement of joining together Micro Array Technology with the laboratory. It is mind boggling. This amazing technology allows us to routinely include *millions* of DNA probes on a *single glass chip the size of a dime*.

Just a few months ago, our Foundation entered a 3-way partnership with the National Human Genome Research Institute at the NIH, and Affymetrix, the company that invented and developed GeneChip Technology. Vicki and I had a chance to visit with Affymetrix at their California Headquarters, and let me tell you what we saw.

We were introduced to a gene chip scanner that generated a computerized array of letters and numbers representing a cluster of genes.

The technicians then scrolled through thousands of markers to find a genetic mutation—the piece of DNA that is scrambled. Using their specialized “cutting edge” technology, they could then unscramble the data, positively identify the defect, and pinpoint its exact location. They could also show us its absence—which was described to us as a “no call” on a particular chromosome—a piece of DNA missing, resulting in a partial deletion of a gene. Amazing new technology, new science, new medicine.

Now, we hope to apply this technology to confirm the TREC test for newborn screening of Severe Combined Immune Deficiency. That test currently produces a remarkable 98.4% accurate result, but with 4 million newborns in the US each year we need a confirmatory test. With a clear diagnosis of SCID, bone marrow transplants now have a better than 90% cure rate. Cure—not a gradual or subtle improvement, which in itself would be a great achievement, but a cure for a newborn with a fatal disease.

Beyond that, there is clear and overwhelming evidence that it is *cost effective* to match up laboratory screening, microarray technology, and transplantation, especially if diagnosed early.

Just a few weeks ago I received—the first ever gene chip for SCID.

So here we are, now able to *screen* for a life threatening disease with *at least* 98.4% accuracy, *treat* with 95% cure rate or better and offer a *cost effective* therapy. It goes on. The Plasma Industry has responded to immunodeficient patients with newer, safer, purer, higher quality immunoglobulins that are effective and have dramatically improved patients' quality of life. We are beginning to realize the long awaited promise of gene therapy.

There are already successes curing immunodeficient children in France, England, Italy and Switzerland covering a wide variety of genetic defects. We have met with many of the families in these countries, and have had an opportunity to embrace their perfectly healthy children. It may be the early stages for gene therapy, but for us it was overwhelming and miraculous to see children perfectly healthy.

All of us are aware of the enormous potential of stem cell research, giving us the possibility for regeneration of tissues, and prospects to cure a wide range of diseases. The science and new discoveries in stem cell research will move forward relentlessly, regardless of anyone's political agenda.

As we enter the most explosive period of scientific discovery ever, everyone can impact on advancing science to improve lives. How does one do this?

We believe there are three very practical initiatives well within our control; these are principles consistent with the treasured advice we received from Bob Good and Bibi Day.

First, we need an unwavering commitment to research—basic and clinical—from the mouse model through translational research to clinical trials and finally to the bedside. We all know that research is narrowly targeted, but so often, discovery erupts entirely by accident—emerging through serendipity. Invariably, that unexpected discovery will lead to disease prevention and treatment.

Second, we should encourage and support new technology that can target disease, improve diagnosis, help develop new protocols and personalize treatment. We should encourage integration into our healthcare system of these new tools that can be brought to the bedside.

Finally, we should commit our support to the most advanced academic medical centers in the world, pursuing excellence wherever that resides.

If an organization or an individual truly believes in the work of a great research institution, a medical school, its affiliates, a department, a division, an investigator, a researcher, or a professor, one must add commitment to the work. One must actively advocate for that research and provide meaningful support. A donor should tell friends and family and colleagues about “cutting edge” advances they are witnessing. Even members of the US Congress want to know more about new scientific developments. After all, it is they that appropriate funds for research at our National Institutes of Health, and we should keep them fully informed about important new research.

We have an opportunity to advance science, accelerate technology, and propel discoveries affecting a wide range of diseases. Now is our time to produce great accomplishments, *inspired* by new technology, *expressed* by brilliant investigators, and *harmonized* by our collective support.

Discovery will flourish, and we will provide hope, enrich lives, create dreams, and rejoice in the most exciting period of scientific discovery ever. Let us go forward together. We thank you for this wonderful honor to come before you at this first Robert A. Good Symposium.