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Dr. Stephen C. Groft, Director
Office of Rare Disease Research
National Institutes of Health
9000 Rockville Pike – Mail Stop 7518
Bethesda, MD 20892

Dear Steve:

I enjoyed meeting you at NIH's Advancing Rare Diseases Research Through Networks and Collaboration conference in Bethesda. I have two suggestions that you might be able to use:

1.) A Rapid-Learning International Health System

I suggest that Dr. Frances Collins (assuming that he is confirmed as NIH Director) schedule an early meeting with his counterparts in large nations with national health systems (e.g., China, India) to create strategic plans for a rapid-learning international health system. These new partnerships will involve linked reference databases (in translatable formats) in participating countries. They will extend the Obama Administration's commitment to a new dimension of electronic health records (and public databases with genomic, protein, behavioral, environmental, and other variables for *in silico* research) into a rapid-learning global system.

2.) A Priority for Rare Diseases

A logical step is to begin with rare diseases, since all participating countries will receive immediate and practical benefits. Once translatable codes are established, physicians in China or India, for example, will receive a pop-up menu whenever a

rare disease diagnosis or billing code is entered. The menu will inform them about international disease networks, patient registries, clinical trial options, and the latest research concerning treatment. Currently (as you know) most rare diseases are treated by off-label medications and guesswork – and the new international system can quickly, by statistical analysis of growing international databases, tell physicians about the range of treatments being used and the options that get the best results.

It is risky to extrapolate US disease rates before knowing the cause of rare diseases. Assuming a cautious 5% estimate for these 6,000+ diseases (defined as less than 200,000 known cases in the US), the 2.4 billion people in China and India alone imply that N=120 million+ patients and their physicians could benefit directly and quickly from these new networks and NIH leadership.

Reference Databases and a 24x7 Global Research System

Concerning Dr. Collins'/NIH leadership for a rapid-learning international health system:

Internationally, there is a mistaken impression that the large number of variables discovered in genomic, protein, environmental, behavioral (etc.) research will require electronic records from hundreds of millions of patients and expenditures – prohibitive in many countries - of billions of dollars. But researchers and statisticians at Kaiser and the RWJ Foundation have selected N=10,000 high quality patient records as the size of a basic US reference database for biomedical research (including sub-samples of ethnic and immigrant sub-populations to contribute to international analysis of gene x environment/cultural interactions). And N=10,000 would be doable for China or India – 100 fully documented cases with high quality data from each of 100 hospitals.

Two further ideas:

- The wider benefits of efficiency. As you may know, the World Academy of Art and Science has been considering how, in its plan for the next five years, it might help to network ideas for a rapid learning international health system. At a recent meeting in Hyderabad, the former President of India urged the WAAS Executive Board to help with rapid learning about energy efficiency – i.e., because, in poorer countries like India, any improvements in energy efficiency will make a disproportionate contribution to their own people and economies. The same case applies to disproportionate benefits from the *in silico* international research system for evidence-based medicine and cost-savings.

- Public resources for science-trained individuals. At the NIH conference I was struck, as a social scientist, by the contrast between the many years of training that researchers spend to think about the cause of diseases and the extraordinary daily requirements to spend time on the logistics of human systems, grant applications, etc. By comparison, the new databases for *in silico* research will add a new dimension that allows researchers, 24 x 7, to use the Internet to think about the cause of diseases and test hypotheses at the speed of thought. If you create public, Internet accessible reference databases, and on-line analysis software and computing capacity, there will be an extraordinary number of physicians and science-trained individuals, worldwide, who can participate.

With my best regards,



(Dr.) Lloyd S. Etheredge, Director
International Scientific Networks Project

cc: Dr. Christopher Austin, M.D. (Director, NIH Chemical Genomics Center)
Dr. Larry Ng, M.D., Dr. Pushpa Bhargava (WAAS Board)