

NIH Coalition to Speed Disease Target Validation

The National Institutes of Health (NIH), 10 pharmaceutical companies, and 8 nonprofit organizations announced on February 4 the launch of a novel partnership to transform the current model for identifying and validating the most promising biological targets of disease for new diagnostics and drug development. Called the Accelerating Medicines Partnership (AMP), the coalition will identify biologic targets of disease most likely to respond to new therapies and will characterize biomarkers with clinical promise. Through the Foundation for the NIH (FNIH), AMP partners will invest more than \$230 million over 5 years in initial projects focusing on Alzheimer disease, type 2 diabetes, rheumatoid arthritis, and systemic lupus erythematosus (SLE). A critical and groundbreaking element of the partnership is the agreement that data and analyses generated will be made publicly available to the biomedical community. The 3–5-year, milestone-driven pilot projects in these disease areas could set the stage for broadening AMP foci to other diseases and conditions.

“Patients and their caregivers are relying on science to find better and faster ways to detect and treat disease and improve their quality of life,” said NIH Director Francis S. Collins, MD, PhD. “Currently, we are investing a great deal of money and time in avenues with high failure rates, while patients and their families wait. All sectors of the biomedical enterprise agree that new approaches are sorely needed. The good news is that recent dramatic advances in basic research are opening new windows of opportunity for therapeutics. But this challenge is beyond the scope of any one of us and it’s time to work together in new ways to increase our collective odds of success. We believe this partnership is an important first step and represents the most sweeping effort to date to tackle this vital issue.”

AMP has been in planning stages for more than 2 years, with participation from scientists in the public and private sectors, progressive refinement of goals, strategy development support from the Boston Consulting Group, and scientific project and partnership management by the FNIH. Through this effort, AMP partners have developed research plans and are sharing costs, expertise, and resources in an integrated governance structure that enables contributions to science from all participants.

Initial research goals for each focus area include:

- Alzheimer disease: (1) Identify biomarkers that can predict clinical outcomes by incorporating an expanded set

of biomarkers into 4 major NIH-funded clinical trials (to include industry support) designed to delay or prevent disease. (2) Conduct large-scale, systems biology analyses of human patient brain tissue samples with Alzheimer disease to validate biologic targets that play key roles in disease progression, as well as to increase understanding of molecular networks involved in the disease as a way to identify new potential therapeutic targets.

- Type 2 diabetes: (1) Build a knowledge portal of DNA sequencing, functional genomic and epigenomic information, and clinical data from studies on type 2 diabetes and its heart and kidney complications. The portal will include existing data and new data from studies involving 100,000–150,000 individuals. The rich collection of curated and collated information in this portal will provide an opportunity to identify the most promising therapeutic targets for diabetes from the growing amounts of potentially relevant data. (2) Focus on DNA regions that might be critical for development or progression of type 2 diabetes and search for natural variations in targeted populations that might predict the likelihood of success of drug development aimed at these targets.
- Rheumatoid arthritis and SLE: (1) Collect and analyze tissue and blood samples from individuals with rheumatoid arthritis and SLE to pinpoint biologic changes at the single-cell level, to facilitate comparisons across the diseases and provide insights into key aspects of the disease process. (2) Identify differences between rheumatoid arthritis patients who respond to current therapies and those who do not and provide a better systems-level understanding of disease mechanisms in rheumatoid arthritis and lupus.

Government members of AMP include NIH and the U.S. Food and Drug Administration. Industry members include AbbVie, Biogen Idec, Bristol–Myers Squibb, GlaxoSmithKline, Johnson & Johnson, Lilly, Merck, Pfizer, Sanofi, and Takeda. Nonprofit organizations participating include the Alzheimer’s Association, American Diabetes Association, Lupus Foundation of America, FNIH, Geoffrey Beene Foundation, PhRMA, Rheumatology Research Foundation, and USAgainstAlzheimer’s. More information about the program and the disease research plans can be found at www.nih.gov/amp.

National Institutes of Health