Ideas for Renewing American Prosperity

If you could propose one change in American policy, society or culture to revive prosperity and self-confidence, what would it be and why?

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Unleash Molecular Medicine

By Peter W. Huber

In the past three decades, drug designers have learned how to craft molecules that modulate specific molecular targets—hence "personalized medicine" that fits precisely targeted drugs to patient-specific molecular profiles. Now, rapidly emerging are literally personal treatments created by reprogramming the genetic code in the patient's own cells.

Scientists have recently developed precise tools for adding, deleting or replacing genes inside live cells—tools that can do in hours or days what took months or years using other gene-editing tools. Reprogrammed stem cells—the progenitor cells that spawn all the rest of our cells—have the unique potential to provide complete cures for a wide range of currently incurable disorders, most notably the thousands of rare but often deadly diseases caused by hereditary genetic factors. Immune-system cells reprogrammed to attack cancers and other diseases have shown enormous promise in early trials.

Unlike conventional drugs, human cell therapies can be synthesized from scratch, one patient at a time, with tools compact and cheap enough to land in hospitals, clinics or laboratories that serve doctors in private practice. The technologies can be used to generate, at relatively low cost, a limitless number of biochemically distinct therapies precisely tailored to the individual patient's needs.

Washington's drug-approval process, grounded as it is in a one-size-fits-all perspective on how drugs are supposed to operate, and anchored in clinical-trial protocols and statistical methods developed decades ago, is lagging far behind the science. We need a regulatory process that can keep pace with a rapid proliferation of highly customized therapies that are grounded in a mechanistic understanding of molecular biology. This will require fundamental changes in clinical-trial protocols and in the type of evidence that is required for drug approval.

Mr. Huber is the author of "The Cure in the Code" (Basic Books, 2013).