

GETTING IT GOLDBLOCKS JUST RIGHT: SCIENCE, REGULATION AND ETHICS

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There are few if any technologies, tools or devices untouched by debates about the proper role of government regulation. From garden-variety pharmaceuticals and biosimilars to electronic health records and decision support systems to human cell therapy/products, the debates variously serve to enlighten, inform, confuse or infuriate.

The bad news is that they also seem to overlook an important consideration at the core of public policy related to new medical interventions: They overlook the fact that bad, clumsy or inapt regulation is a fat target. While it is easy to hit, doing so should provide comparatively little satisfaction.

No reasonable person would seriously argue in favor of any of the following propositions:

- All drugs and devices must be risk-free.
- Patients should have no say in the regulatory process.
- Physicians should have no say in clinical applications of new treatments.
- It is laudable when treatments believed to be safe and effective are impeded.
- It is laudable when treatments believed to be unsafe and ineffective are expedited.

Let us further assume for the sake of discussion that no one will seriously endorse a completely laissez-faire system – that is, one with no regulation at all. Even the most committed libertarian is likely unwilling to permit her mother to be a subject in an experiment with no Institutional Review Board (IRB), no Data Safety Monitoring Board (DSMB), no review and no oversight of the substances injected into her body. Let us also agree that an overbearing, oppressive, draconian and inefficient regulatory system is equally unacceptable. Even the most committed statist is likely unwilling to have his mother suffer from a lack of creative and health-enhancing science caused by bad regulation.

The challenge before is whether and how we can get it “Goldilocks just right” – not too big and not too small, too hard or too soft ... neither too heavy-handed nor too hands-off.

Open society thrives on controversy. Not controversy for its own sake, which can be shrill and fatuous, but controversy because the clash of well-wrought ideas has tended to produce better ideas. What Ricordi calls “constructive debate”¹ is as good a start as any, and the issues and topics he itemizes all cry out for more debate and, as important, more science: To what extent ought the FDA to prevent physicians from treating patients with the patients’ own stem cells? How should cellular therapies be regulated? Would weakening regulatory standards increase patient risk? There are many others². The simple itemization of these questions is a necessary condition for the advancement of debate.

In addition to the too much/too little debate, we also need to consider what a Goldilocks-grade regulatory system might look like and, as important, why we are reluctant to support it. That is, we should imagine how a well-funded FDA would operate. Would it use additional resources to regulate more, or would it simply do a better job? Remember, for every patient advocate righteously and correctly aggrieved by a regulatory system that seems to be too slow or too heavy handed when it comes to human cell-based therapies, there is a patient advocate righteously and correctly aggrieved by a regulatory system that has allowed synthetic mesh to become part of the surgical armamentarium with precious little evidence³.

Put differently, it’s time to give more serious consideration to the idea that a well-funded regulatory apparatus might actually have the resources to do a better job, and that that might consist in better and more creative ways of ensuring safety without stifling progress.

Compare in this regard the suggestion for electronic health records and medical software that one might regulate not the actual software, line-of-code-by-line-of-code, but, rather, at the larger-grained level of system functionality⁴.

Make no mistake, (the fear of) increased regulation will always have a chilling effect if the regulation is seen or believed to be faulty, wrong or misguided. In fact, though, we have witnessed extraordinary progress in biomedical research even with much-maligned oversight and regulatory systems. Regulation itself is not bad. The idea that better-sourced regulators will themselves improve quality has never been put to the test. Constructive debate surely requires it, and patients deserve nothing less.

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