Comment

Doctopic: Analysis and Interpretation

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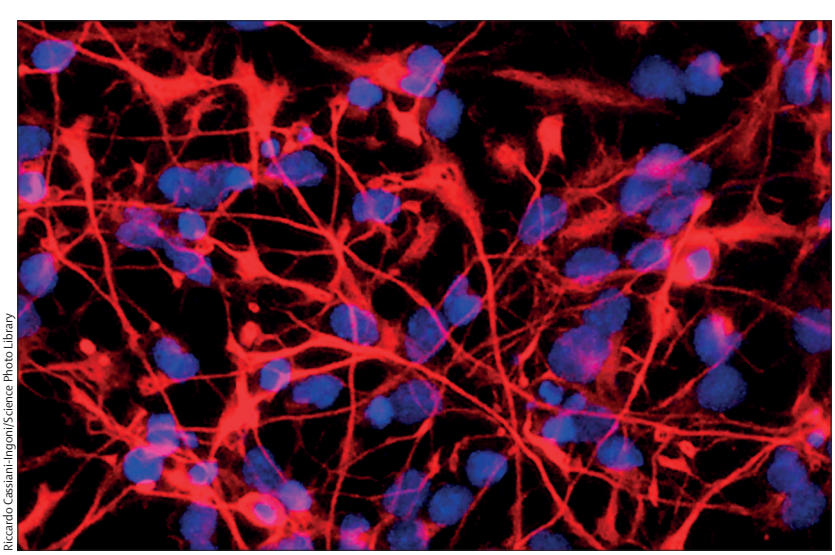
Regenerative medicine: challenges and opportunities

In 2018, the *Lancet* Commission on stem cells and regenerative medicine[1] reported how, despite an exponential growth in experimental therapies, there had been limited clinical uptake. Regenerative medicine comprises various novel approaches such as cell and gene therapy that have produced life-saving therapies for a few genetic diseases affecting blood or skin. Enthusiasm about the broad potential of regenerative medicine led to a gap between expectations and the realities of translating technologies into clinical practice. The *Lancet* Commission called for rethinking to tackle the combination of problems residing in poor-quality science, unclear funding models, unrealistic hopes, and unscrupulous private clinics.[1]

What has happened since then? On June 3, 2020, *Challenges and Potential in Regenerative Medicine*,[2] a report by the European Academies Science Advisory Council (EASAC) and the Federation of European Academies of Medicine (FEAM), was published to raise awareness of the opportunities and challenges of regenerative medicine for the scientific community, regulators, health services, and public policy makers, and to provide recommendations to inform EU strategy.

The pace of science continues to advance rapidly and the EASAC–FEAM report recommends sustained investment from basic to clinical science to provide resources for innovation. While opportunities are considerable in many fields, such as neurological or metabolic disorders, challenges grow. One problem continues unabated: commercial clinics offer unregulated products and services that promise a wide range of benefits but use poorly characterised treatments with little evidence of effectiveness, potential safety concerns, vague scientific rationale, and the primary intention of financial profit.[3] The EASAC–FEAM report describes several principles (eg, clear and accessible evidence of clinical efficacy) to inform patients who are contemplating such offerings. In Europe, a crucial criterion for patients in deciding whether to participate in a novel clinical trial is that they should not be expected to pay clinical research costs.[2]



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Another problem is an evidence crisis[4] resulting from premature marketing approval and commercialisation of expensive approaches, facilitated by regulatory authority initiatives for accelerated access.[5] The *Lancet* Commission posited a scenario where it was possible to distinguish between "good" and "bad" clinical activity.[1] However, making this distinction is becoming increasingly difficult because companies might start from a reasonable hypothesis, collect some evidence, and publish in reputable journals, but data can be inflated and insufficiently replicated while risk and benefit are inadequately ascertained.[6] The EASAC–FEAM report[2] advises that, in an era of international competitiveness when some regulatory frameworks have become increasingly permissive,[7] it is essential that the EU does not lower its regulatory threshold without assessing the consequences for patient safety, health-care budgets, and public trust in science.

Alongside ensuring regulatory procedures are robust, transparent, and evidence-based while still being rapid and accurate, there is much else to be done. Priorities highlighted by the academies' consensus include: reinvigoration of EU research infrastructure, particularly for translational and clinical research;[8] support for new models of partnership between academia and industry while ensuring ethical development;[9] inserting regenerative medicine in curricula for medical education and professional training;[10] alerting against non-peer-reviewed "predatory" journals;[11] developing health services' institutional readiness in relation to regenerative medicine research; and engaging with the public and patients to counter misinformation.

The challenges are difficult to tackle when some patients have no other therapeutic option but to resort to stem cell clinics or products given conditional marketing status on the basis of inadequate evidence. Nonetheless, the advent of a new European Commission brings additional opportunities to protect patients by harmonising regulatory frameworks and guide the public to equitable and safe access to new but reliable therapies while educating the next generation of professionals.

The EASAC and FEAM are now working at global scale through the InterAcademy Partnership to ensure support for responsible research and innovation in regenerative medicine. The recommendations of the EASAC–FEAM report will help to inform this process, with the hope that the recommendations provided, and commented on here, will help to achieve a rapid but safe development of regenerative medicine.

We all contributed to the EASAC–FEAM report discussed in this Comment. GC has a patent (WO2007093412) issued. RF, GG, and VtM declare no competing interests.

Giulio Cossu, Robin Fears, George Griffin, Volker ter Meulen
giulio.cossu@manchester.ac.uk

Division of Cell Matrix Biology and Regenerative Medicine, University of Manchester, UK (GC); European Academies Science Advisory Council, German National Academy of Sciences Leopoldina, Halle (Saale), Germany (RF, VtM); and Institute of Infection and Immunity, St. George's, University of London, London, UK (GG)

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