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Putting Translational Science onto a Global Stage

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Abstract

Global collaboration in translational science promises to accelerate the discovery, development and dissemination of new medical interventions. In this article, we introduce a new international collaboration of translational science organizations and highlight our initial strategy to reduce or remove bottlenecks in translation. A first step in this process is increasing the awareness and understanding of the field among key stakeholder groups.

There is broad consensus that the development of new medical interventions (drugs, devices, diagnostics, vaccines, behavioral interventions, etc.) simply takes too long, costs too much, and too often ends in failure. Many of the recent reviews (1–4) of the drug discovery enterprise provide the sobering statistics and illuminate the decline in biopharmaceutical productivity over the past few decades. The "innovation gap" between fundamental discoveries in human health and disease and the delivery of new therapeutic options to patients has remained stubbornly persistent. However, every challenge brings tremendous opportunities to develop innovative solutions to overcome them. The field of translational science is poised to provide solutions to those barriers and help bring more medicines to more patients more quickly. This article serves to increase the awareness of the burgeoning field of translational science as a unique discipline, and introduce a nascent international collaborative effort in this arena.

Defining translation and translational science

Translation is defined as the process of turning observations in the laboratory, clinic and community into interventions that improve the health of individuals and the public — from diagnostics and therapeutics to medical procedures and behavioral changes. The central challenge of the translational process is to advance basic discoveries into tangible health benefits. However, this process is poorly understood scientifically and subject to frequent dislocations organizationally, explaining in large part the high failure rate of translation and

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dictating the need for systematic study. Thus, translational science is defined as the field of investigation focused on understanding the scientific and operational principles underlying each step of the translational process. This broad interpretation of translation and translational science reflects the dynamism of the process whereby information gathered at each step informs others before and after it.

A new player arrives

The rapid growth of translation over the past decade and a half has been aided by the development of ever higher throughput technologies, the identification of thousands of new therapeutic targets through advances in human genomics and proteomics, and improvements in predictive efficacy and toxicology models for drug development, to name just a few. Many novel therapeutic horizons have come into view along the way, including cancer immunotherapy, gene editing, stem cell therapy, and precision medicine.

Running counter to this expansion has been continued reduction of effort by the biopharmaceutical industry in the early pre-clinical therapeutic discovery space. Coupled with seemingly intractable challenges at all stages of translation that no one company or institution was poised to tackle, these conditions presented an opportunity for publiclyfunded organizations to establish dedicated translational science centers. Realization that a new type of organization was needed, to serve as an "adaptor" and "de-risker" between basic research and commercial organizations, led to the creation of dedicated translational science efforts in multiple countries. These new translational players fill the gulf between basic discovery and commercial application, and advance translation as a scientific discipline for the benefit of all stakeholders, public and private. Between 2000 and 2011, governments, non-profit organizations and researchers in the European Union, Canada, Australia and the United States recognized the value of building their national and regional translational capacity and supported the creation of such dedicated translational research organizations. MRC Technology (MCRT) in the UK, the European Infrastructure for Translational Medicine (EATRIS), The Centre for Drug Research and Development (CDRD) in Canada, Therapeutic Innovation Australia (TIA), and the National Center for Advancing Translational Sciences (NCATS) in the US [Supplementary Box 1] were built to catalyze the translational process and accelerate therapeutic innovation.

Despite their geographic diversity and somewhat country-specific mandates, our organizations approach translational science with a shared mindset and use common language to describe the nature of the opportunities and challenges in the field. There is also consensus among our organizations concerning the need to raise the level of awareness, understanding and appreciation of translation and translational science among scientists and the public. Working together will better enable this promising field of study to accelerate the development of medical interventions that improve individual and public health. The organizations therefore agreed to establish a collaborative effort to leverage the experience, expertise and credibility of each and promote translational science with a unified voice on a global stage. The audiences that must be engaged are basic scientists, funders and policy-makers, industry, and patients and their associated community members.

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Increasing awareness

Basic scientists provide the foundation for translational development and are therefore an essential component of the translational science ecosystem. However, researchers focused on fundamental mechanisms are often not aware of the nature and complexity of the steps required for a promising discovery or technology to advance to the clinic. Only a small percentage of projects from traditional academic and non-profit research laboratories have progressed to a point sufficient to incentivize and attract the capital investment necessary to enter the pre-clinical and clinical testing phases. To address this failure to progress, which has caused many promising discoveries to lie fallow, each of our organizations provide collaborative scientific and technical expertise and resources to basic researchers to "derisk" discoveries to a point where a biopharmaceutical company can either be formed or will license the technology for further development. For those basic scientists who wish to see their discoveries advance to the clinic, acquiring a thorough understanding of the processes, core competencies, and technical and financial resources for this purpose as well.

Policy makers and funders in each of our countries are commonly frustrated by the evident mismatch between the breathtaking pace of basic scientific discovery and the meager rate of substantial health improvements. They have often heard of the "valley of death" in biomedical research, but may not be aware that the field of translational science exists to remedy it. There is therefore an urgent need to increase the understanding of translation among funders and policy-makers and the substantial contributions it makes to public health and the economy. The value of continued investment in this field, as well as the greater impact it could have with increased resources, needs to be more consistently communicated on a regional, national and international level to funders and policy-makers. By leveraging its global scope and presenting a unified front of leading translational scientists from nonprofit organizations and government institutions, this global collaborative hopes to communicate critical information to funders and policy-makers and engender greater recognition and appreciation of the field.

Patients, disease advocacy groups and associated community members are integral participants of any translational science team. Translational researchers can learn invaluable information about disease symptomatology and therapeutic priorities from the real-world experiences of patients living with a disease. Involving them in each stage in the development of a new medical intervention will help catalyze the entire process. Likewise, patients can more effectively engage in and derive value from translational science when they are informed of how the translational science ecosystem operates. This global collaborative will work to further the paradigm by which patients and scientists develop new treatments more rapidly by working together.

A final challenge facing translational science is attaining full recognition as an independent discipline with unique requisite knowledge and skill sets. While translational science is multidisciplinary by nature, it is greater than the sum of its individual parts and requires the integration of the experiences and information from a variety of disciplines into a single practice. The well-trained translational scientist, therefore, may be an expert in a particular

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aspect of translation (such as pharmacology or epidemiology) but also possesses essential knowledge on the full spectrum of translation and uses it to directly inform his or her research program. The work of such scientists has led to the repurposing of existing drugs to treat new diseases, the shortening of the time it takes to launch clinical trials, the development of innovative technologies to identify promising therapeutic targets, and the creation of public-private partnerships to de-risk early-stage drug candidates. By encouraging the recognition of translational science as a formal field of study, this collaboration hopes to increase the number and diversity of practitioners in the translational workforce.

Looking to the future

Challenges in the reproducibility of pre-clinical translational research, the exceedingly high failure rate of promising new interventions, the lack of scientific understanding or efficient processes throughout the translational process, and uncertainties in the policy and funding landscape all extend beyond geographic borders. No one organization operating in isolation is likely to overcome all of these complex barriers; a concerted international approach is necessary. The organizations represented in this collaborative will take advantage of opportunities to coordinate our endeavors, leverage our combined expertise and resources, share best practices, broadly disseminate our results, and present a unified voice calling for greater recognition of the contributions and support of translational science.

In conclusion, the promise and challenges of the new field of translational science, including those in drug discovery and development, must be brought to the forefront for all stakeholders in the health research system. These stakeholders include patients and patient advocacy groups, non-profit foundations, politicians and policy makers, funders, academic researchers and research institutions, and pharmaceutical and biotechnology companies. Integrating and leveraging the cumulative knowledge of translational organizations on a global level promises to more rapidly advance innovative medicines to patients in the myriad disease areas with high unmet medical need, leading to improved health outcomes for patients, more sustainable healthcare systems, and overall benefit to society.

Supplementary Material

Refer to Web version on PubMed Central for supplementary material.

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