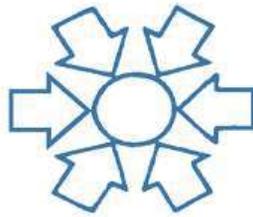


Overview: Translational Biomedical Research Funding in Advanced, Market-Based Economies



Matthew Shaw

&

Drake Palmer

Federal Financial Analytics, Inc.

1140 Nineteenth Street, NW

Washington, D.C. 20036

info@fedfin.com

www.fedfin.com

August 22, 2018

Purpose and Initial Considerations

This paper¹ assesses translational research funding provided by governments in advanced, market-based economies. Translational biomedical research receiving this funding is defined as research from the pre-clinical to late-stage patient trial period, the point at which venture capital or biopharma-company funding steps in. It thus looks at the extent to which public funding partners with or even usually steps in for private capital to speed translational projects past the “valley of death” that now keeps much promising basic research from advancing quickly to treatment and cure. Translational funding is not defined here to include government support for organizational, mentoring, or similar programs that do not directly support actual translational trials.

Executive Summary

Although the United States is the global leader in private-sector biomedical research, it is significantly behind many of its peers when it comes to one critical component of these efforts: public funding for translational research. Translational biomedical research builds on the basic science conducted in the U.S. largely through the National Institutes of Health (NIH) funded by U.S. taxpayers, applying successful basic research to actual treatments and cures that enhance health and well-being. Basic science has significant public funding in the U.S. – NIH’s FY2018 budget was over \$36 billion,² with a large percentage of this dedicated to basic science – but total public funding for all aspects of translational research – including support functions – in the same year totaled less than \$1 billion.³ The advanced, market-based economies surveyed in this paper largely provide direct funding support to translational biomedical research in greater amounts than the U.S. on a percentage of gross domestic product (GDP) basis and, often, also on a raw dollar-total basis. Furthermore, programs in the surveyed countries appear to be significantly more targeted than those in the U.S., with most jurisdictions operating translational research initiatives aimed at specific disease groups as well as supporting translational research more broadly.

For the purposes of this paper, government funding that is directed to support translational research but is not spent on actual research (i.e., funds for administrative costs) has been excluded from spending totals with limited exceptions as noted. Efforts have been made to quantify the percentage and/or amount of funding that goes directly to translational research in programs which budget direct and indirect translational research spending together. All currency conversions reflect August, 2018 values.

¹ This paper is a *pro bono* work designed to place pending legislation in the U.S. House of Representatives (H.R. 6421, The Faster Cures and Treatments for Eye Diseases Act) that would create a new translational research funding guarantee from the Federal government in the broader context of translational research funding programs in advanced, market-based economies. It is not intended to be a comprehensive, peer-reviewed examination of different countries’ approaches to translational research funding and serves only to show comparisons broadly.

² National Institutes of Health (NIH), “What We Do: Budget,” last modified April 11, 2018, <https://www.nih.gov/about-nih/what-we-do/budget#note>.

³ NIH, “National Center for Advancing Translational Sciences, About the Center: Budget,” last modified July 10, 2018, <https://ncats.nih.gov/about/center/budget>.

The United States

Publicly funded translational research in the U.S. is housed in the National Center for Advancing Translational Sciences (NCATS), a division of NIH. The FY2018 budget for NCATS totaled \$742.4 million, \$542.8 million of which is appropriated specifically to NCATS' Clinical Translational Science Awards (CTSA) program. However, almost all of the funding for these programs goes not towards translational research for specific diseases, but instead towards research addressing commonalities underlying all areas of medicine as well as connecting researchers and institutions to share their expertise and resources.

The United Kingdom

U.K. research and innovation efforts were subject to a major overhaul in 2018, with nine formerly independent agencies now under the umbrella of a new agency, U.K. Research and Innovation (UKRI). One of these formerly-independent agencies, the Medical Research Council (MRC), is one of the two primary channels through which the U.K. funds translational biomedical research. With a 2016/2017 budget of £755.5 million⁴ (\$962 million) the MRC funds translational research primarily through grants to researchers in universities, medical schools, and research institutes. Project funding exists at multiple levels of the translational path: support for initial steps, pre-clinical development, early clinical testing (including with regard to repurposing existing therapies for new use), and late-stage clinical evaluation. Specific translational-support programs housed in the MRC include:

- the Biomedical Catalyst (BMC) Confidence in Concept scheme,⁵ which provides awards of £250,000 to £1.2 million (\$321,500 to \$1.54 million) to institutions to provide support for the earliest stages of translational research projects. Funds are intended to accelerate the transition from discovery research to translational development via support efforts showing the viability of a translational research project;
- the BMC Developmental Pathway Funding Scheme, which funds proof of concept trials, including early clinical testing of novel therapeutics, devices, and diagnostics;
- the Efficacy and Mechanism Evaluation Program, which includes clinical trials and evaluative studies to determine the clinical efficacy of interventions where proof of concept in humans has been previously achieved; and
- effectiveness trials designed to measure the effectiveness, costs, and broader impact of health technologies for those who use, manage, and provide patient care.

The other primary channel through which the U.K. funds translational biomedical research is the National Institute of Health Research (NIHR), which remains outside of the newly-formed UKRI and is the

⁴ U.K. Research and Innovation, "Medical Research Council, What we do: Facts & Figures," accessed July 11, 2018, <https://mrc.ukri.org/about/what-we-do/spending-accountability/facts/>.

⁵ U.K. Research and Innovation, "Confidence in Concept scheme 2017," accessed August 9, 2018, <https://mrc.ukri.org/funding/browse/confidence-in-concept-scheme-parent/confidence-in-concept-scheme-2017/>.

primary research arm of the National Health Service (NHS). In addition to partnering with the MRC on efforts such as the Efficacy and Mechanism Evaluation Program, the NIHR operates several programs designed to advance translational biomedical research. Biomedical Research Centers are at the forefront of these efforts, with twenty centers now set up to conduct translational research in specific categories (e.g., cardiovascular, mental health, cancer, vision). Funding across all twenty centers totals over £816 million (\$1.04 billion) for five years.⁶

The European Union

Billed as the world's "biggest public-private partnership in the life sciences," the EU's Innovative Medicines Initiative⁷ is a €5.3 billion (\$6 billion), 13-year initiative focused on providing funding to areas of translational research with major unmet needs. Funding for the initiative is split 50-50 between the public and private sector, with the EU's Horizon 2020 program contributing one euro in public funds for every one euro raised by private investors. Specific objectives of the program include:

- developing new therapies for diseases for which there is a high unmet need (e.g., Alzheimer's) and limited market incentives (e.g., antimicrobial resistance);
- reducing the failure rate of vaccine candidates;
- increasing the success rate in clinical trials of WHO-identified priority medicines; and
- reducing the time to reach clinical proof of concept.

The initiative is also intended to increase the efficiency and effectiveness of medicines and treatments.

The EU also operates a translational research support initiative known as the European Advanced Translational Research Infrastructure in Medicine (EATRIS),⁸ which is similar in some aspects to NCATS in the U.S. With an annual budget approaching €2 million (\$2.27 million), this program focuses on providing researchers with the means to develop basic research into viable products. Specifically, funding from this initiative goes towards providing researchers with state-of-the-art facilities, technologies, and translational know-how, as well as training and support services. The initiative also seeks to optimize the research already underway in academia, industry, and the public sector. By reducing informational fragmentation and establishing multidisciplinary teams across the array of clinical, scientific, regulatory, and product development projects, EATRIS aims to ensure that all steps in the treatment-and-cure development process are considered from the start. EATRIS is one of several Biomedical Science Research Infrastructures (BMS RIs) operated under the EU's Coordinated Research Infrastructures Building Enduring Life-Sciences Services (CORBEL) platform.

⁶ National Institute for Health Research (NIHR), "How we are managed: NIHR Biomedical Research Centres (BRCs)," accessed July 17, 2018, <https://www.nihr.ac.uk/about-us/how-we-are-managed/our-structure/infrastructure/biomedical-research-centres.htm>.

⁷ Innovative Medicines Initiative (IMI), "IMI mission and objectives," accessed July 23, 2018, <https://www.imi.europa.eu/about-imi/mission-objectives>.

⁸ European Commission, "EATRIS Report Summary: Final Report Summary – EATRIS (The European advanced translational research infrastructure in medicine)," accessed July 24, 2018, https://cordis.europa.eu/result/rcn/55325_en.html

France

In tandem with the EU's EATRIS initiative, France operates its own translational research infrastructure for innovative therapies directed at medical innovation for the treatment of diseases of the nervous system. NeurATRIS⁹ brings together experts in both the public and private sectors (e.g., hospitals, public research institutes) to accelerate the translation of discoveries in basic research into medical innovations. All work carried out by NeurATRIS aims to accelerate the understanding of both biological and pathophysiological mechanisms and the development of the nervous system, as well as to facilitate completion of pre-clinical and clinical trials. The program provides an uncapped number of grants of up to €50,000 (\$56,700) per grant to academic research laboratories and biotechs (private partners are allowed, but only academics and biotechs are eligible for funding). Successful applicants also receive access to NeurATRIS technology platforms, expertise, and support (e.g., advanced neuroimaging tech, cell and animal models).

France's major translational research initiatives are conducted through its National Institute of Health and Medical Research, better known as Inserm. Founded in 1964 and operating under the joint authority of the French Ministries of Health and Research, Inserm funds biomedical research across the entire range from the laboratory to the patient's bedside. Inserm spent €856 million (\$971 million) in 2016¹⁰ and contributes significant resources towards clinical trials; in 2016 alone, it sponsored 7 phase I trials, 18 phase II trials, 2 phase III trials, and 1 phase IV trial.¹¹ Funding is provided on an approximately 2:1 state-to-external funding basis and approximately 77 percent of Inserm's budget is earmarked for research unit scientific activity, with the remaining 23 percent going towards scientific leadership/support and general expenditures (e.g., administrative costs).¹²

Germany

Although the German government has traditionally provided significant funding for basic biomedical research, it only began a dedicated, federally-funded program for translational biomedical research in 2010. The Health Research Framework Programme, under the authority of the Federal Ministry of Education and Research, was launched to provide strategic impetus for combatting major diseases through the use of long-term translational research.¹³ To date, the Federal Ministry of Education and

⁹ NeurATRIS, "NeurATRIS, translational research infrastructure for innovative therapies in Neuroscience," accessed July 27, 2018, <http://www.neuratris.com/index.php/en/neuratris>

¹⁰ Inserm, "L'Inserm En 2016," accessed August 1, 2018, https://www.inserm.fr/sites/default/files/2017-11/Inserm_RA_2016.pdf.

¹¹ Inserm, "Inserm Presentation Brochure 2017," accessed August 1, 2018, https://www.inserm.fr/sites/default/files/2018-01/Inserm_Plaquette_201707_EN.pdf.

¹² Inserm, "Budget," accessed August 1, 2018, <https://www.inserm.fr/en/about-inserm/missions/budget>.

¹³ Georg Schütte, *Speech at the Federal Ministry of Education and Research, Berlin: The German Centres for Health Research: A Contribution to Effective Translational Medicine* (January 11, 2017), available at <https://www.bmbf.de/de/the-german-centres-for-health-research-a-contribution-to-effective-translational-medicine-3803.html>.

Research has provided more than €13 billion (\$14.74 billion) in support to the program,¹⁴ with a majority of funding going towards the German Centres for Health Research. Six centers are currently in operation, each focused on a particular medical field: cancer, diabetes, cardiovascular diseases, infectious diseases, lung diseases, and neurodegenerative diseases.

Germany is also in the process of reinvigorating its “Excellence Strategy.” This funds a series of “Clusters of Excellence” that support project-based funding at publically-funded universities across Germany. It is expected that between 45 and 50 Clusters of Excellence will be funded with each annually receiving between €3 million and €10 million (between \$3.4 million and \$11.4 million), with indirect project costs capped at 22% of the approved and expended project funds.¹⁵ Total funding is expected to be approximately €385 million (\$437 million) per year across all Clusters. Notably, several translational research projects initiated during the previous iteration of the initiative have already transitioned to the new program (e.g., the University of Cologne’s Cluster of Excellence in Aging and Age-Associated Disorders).

Australia

Australia’s main source of direct public funding for translational biomedical research is its National Health and Medical Research Council (NHMRC). Translation has been a particular focus of the NHMRC in recent years and now is enumerated as the Council’s second highest ranking purpose behind investment in research (which also includes translational research funding capacity). As of the NHMRC’s 2016-2017 report,¹⁶ direct funding commitments under the translation purpose totaled almost A\$810 million (\$586.2 million), with more than A\$173 million (\$125.2 million) specifically committed to translating research. Approximately A\$254 million (\$183.82 million) is committed to building additional capacity for translational research while another A\$382.5 million (\$276.8 million) is committed to creating knowledge within the translational realm. Prominent initiatives funded through these commitments include Australia’s seven Advanced Health Research and Translation Centres (AHRTC), with each receiving A\$10 million (\$7.3 million) in appropriated funding for the 2017-2018 budget. Additional funds are available on a grant basis for these centers; for example, the Minister of Health in 2016 authorized additional funding of up to A\$100,000 (\$72,800) for each AHRTC as a one-off opportunity to apply for additional support to a specific translational research project.¹⁷

Due to Australia’s focus on a comprehensive approach to medical research within specific disease groups, there is a great deal of translational research funding outside of that budgeted specifically for translational research advancement. For example, almost A\$15 million (\$11 million) in funding for

¹⁴ Project Management Jülich, “Health Research Framework Programme,” accessed August 6, 2018, <https://www.ptj.de/en/project-funding/health-research>.

¹⁵ Deutsche Forschungsgemeinschaft (German Research Foundation), “Germany’s Excellence Strategy: Call for Proposals in the Clusters of Excellence Funding Line,” last updated September 30, 2016, http://www.dfg.de/en/research_funding/announcements_proposals/2016/info_wissenschaft_16_59/index.html.

¹⁶ National Health and Medical Research Council, “Annual Report of the National Health and Medical Research Council 2016-217,” October, 2017, available at https://www.nhmrc.gov.au/files_nhmrc/file/publications/17409nhmrc_annualreport2016-2017_0.pdf.

¹⁷ *Ibid* at 58.

clinical research at Australia's Centres of Research Excellence¹⁸ is included in its "investment," not "translational" purpose budget despite constituting a critical stage of translational research. Broadly, Australia totaled over A\$293 million (\$213 million) in expenditures on clinical medicine and science for the 2016-2017 fiscal year and has spent over A\$1.4 billion (\$1 billion) on clinical science and medicine since 2012.¹⁹ Australia also leverages public funding to bring in millions of dollars in private funds, none of which are accounted for in these numbers. Notably, a five-year, A\$354 million (\$257.5 million) joint project between the Australian Government, Queensland Government, The Atlantic Philanthropies, the University of Queensland, and Queensland Institute of Technology led to the creation of the Translational Research Institute of Australia, a state of the art facility that is now the largest medical research institute in the southern hemisphere.²⁰

Canada

Canada is in the process of overhauling its funding of science and medical research after a decade in which the previous government implemented an industry-oriented research approach and cut public funding across all facets of the medical-research lifecycle. Government-funded translational research in Canada is generally included in the funds allocated for full research-cycle projects such as Genome Canada.²¹ Since Genome Canada's creation in 2000, it has received C\$1.1 billion (\$840 million) in government funds and raised over C\$1.6 billion (\$1.22 billion) in additional co-funding commitments from the private sector. The project supports a network of genomic technology and innovations centers that emphasize knowledge translation. Similarly, Canada's Stem Cell Network, which also emphasizes translating research into commercial projects, has received C\$95.3 million (\$72.4 million) in funds since its creation in 2001.

New Zealand

Although New Zealand is home to significant translational biomedical research, the vast majority of this research is housed in New Zealand's university system. The university system received NZ\$526 (\$352 million) in government funding budgeted for research in 2016;²² data on the percentage of this used specifically for translational research is unavailable. Examples of translational research in the university system include the University of Otago's Centre for Translational Cancer Research, Colorectal

¹⁸ *Ibid* at 36.

¹⁹ *Ibid* at 34.

²⁰ Translational Research Institute of Australia, "A state-of-the-art facility," accessed August 16, 2018, <https://www.tri.edu.au/learn/state-art-facility>.

²¹ Canada's Fundamental Science Review, *Investing in Canada's Future: Strengthening Foundations of Canadian Research*, 102 (April 10, 2017), available at [http://www.sciencereview.ca/eic/site/059.nsf/vwapi/ScienceReview_April2017-rv.pdf/\\$file/ScienceReview_April2017-rv.pdf](http://www.sciencereview.ca/eic/site/059.nsf/vwapi/ScienceReview_April2017-rv.pdf/$file/ScienceReview_April2017-rv.pdf).

²² Education Counts, "Government funding of tertiary education," New Zealand Ministry of Education, accessed August 22, 2018, https://www.educationcounts.govt.nz/statistics/indicators/main/resource/government_funding_of_tertiary_education

Translational Research Group, and Centre for Musculoskeletal Outcomes Research, and Massey University's IFS Biomedical Research Centre. New Zealand's Health Research Council has also identified the need to develop new funding models for translational research as a priority to be addressed in its 2017-2027 research strategy.²³

²³ New Zealand Ministry of Business, Innovation and Employment and Ministry of Health, *New Zealand Health Research Strategy 2017-2027: Summary of submissions and consultation*, 9 (February, 2017), available at <https://www.health.govt.nz/system/files/documents/publications/nz-health-research-strategy-2017-2027-summary-submissions-feb17.pdf>.