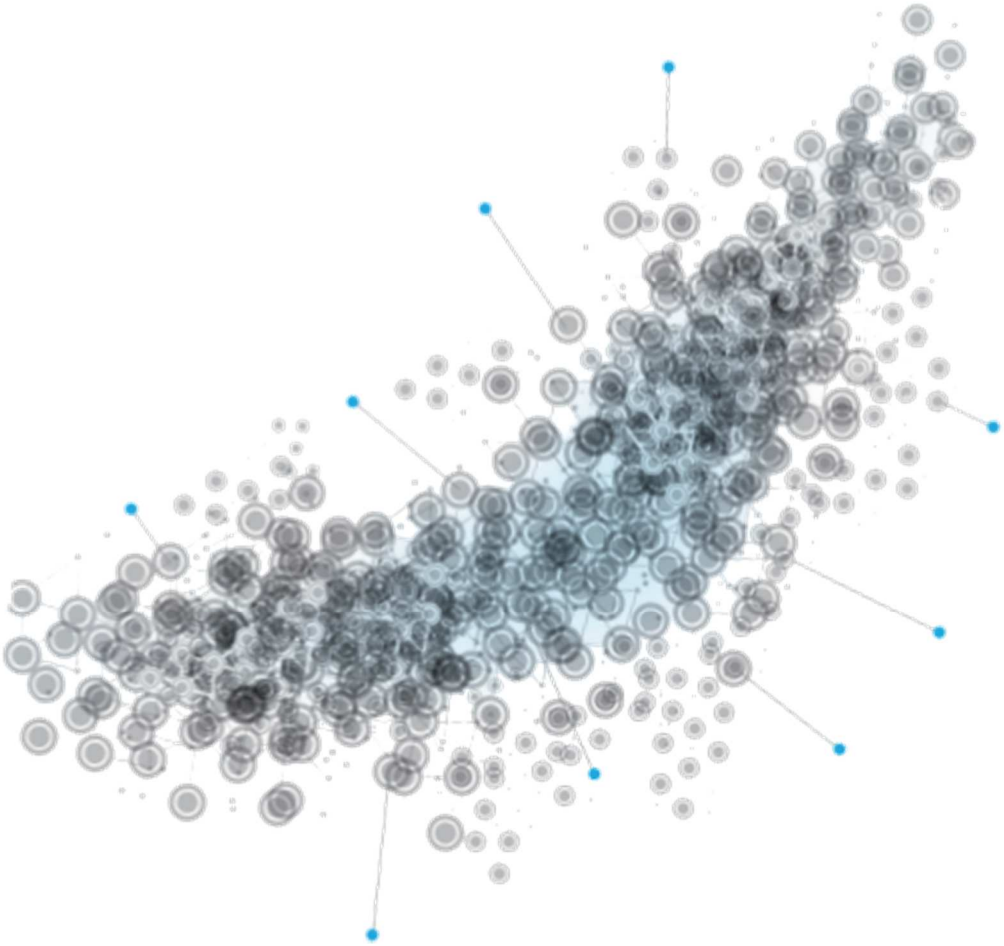
BASTION

ROUNDTABLE

FROM LAB TO CLINIC

Best practices in translational
medicine & innovation management

4th June 2013, European Parliament



Report of the BASTION roundtable

Introduction

“As national health budgets shrink, the expectations of patients are not shrinking”

Jerzy Buzek MEP and former president of the European Parliament in his opening address to experts meeting to discuss the progress and ambitions of the BASTION project, share best practice, and identify gaps and barriers in translational medicine across Europe, at a workshop held in the European Parliament, 4 June 2013

With Europe’s healthcare systems facing the dual pressures of austerity-era cuts and an ageing population, there has never been a more pressing need to establish robust systems for translating rich seams of basic research into the clinic, providing improvements in patient care and generating innovative commercial products.

No one country or clinical specialism can claim to have a defined and tested formula for driving this process of translation, with pockets of best practice spread across Europe. However, it is also the case that the newer member states have further to go in building a framework for medical innovation.

In some senses, this presents an opportunity, since it is possible to draw on the wealth of existing best practice and the evidence base of what works – and what does not.

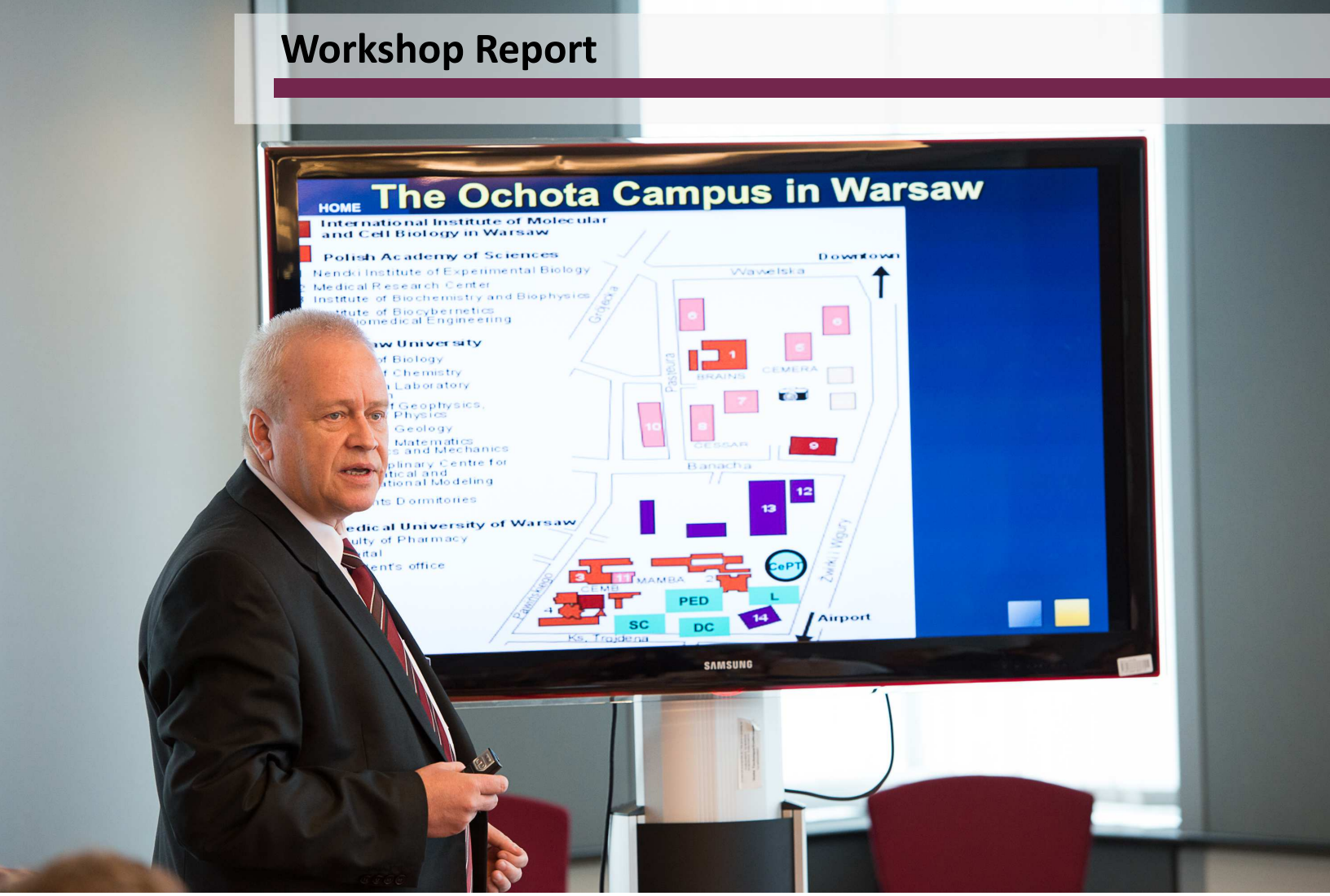
This is the backdrop to a workshop held in the European Parliament on 4 June 2013, at which the leaders of ‘BASTION – From Basic to Translational Research in Oncology’, a €5.3 million project to build up the basic research and translational medicine capabilities of the Medical University of Warsaw, outlined the ambitions of the project and progress to date, and some of Europe’s leading experts provided insights from their experiences on the front lines of translational research.

Executive Summary and Recommendations

Translational medicine does not end with the formation of a spin-out or with getting a product into a Phase I clinical trial. It is necessary to keep going until products get to market and patients get access to them. This can be a long, drawn-out and expensive process and technology transfer and commercialisation systems need to provide support across the piece.

A number of essential elements are required to foster the development of the translational medicine ecosystem:

- The foundation stone is world class research, carried out in world class facilities.
- It is necessary to focus, and to build critical mass. No one institution, or cluster, or region has all the capabilities and collaboration with academic groups in other countries, with biotech and medtech companies, and with pharma, is essential.
- Build human capital – not only in the shape of experts in specific discipline, but scientists who understand the importance of intellectual property, are schooled in entrepreneurship and who relish working in inter-disciplinary settings.
- Universities need to explicitly acknowledge their role as generators of industry-ready knowledge and put in place the facilities and resources required to produce it.
- Provide mentors and role models.
- The scope of translational medicine should be extended to take in patient access. This means that the requirements of regulators, reimbursement bodies and payers must be taken into account at the earliest stages of a research project.
- Focus on patient need. With healthcare systems under severe pressure, innovative and effective drugs and devices that address unmet medical needs, not me-too products are required.
- The pharmaceutical industry is now looking to the early stages of research for innovation to fuel its denuded pipelines. Translational medicine needs to tap into this demand; universities need to advertise their capabilities and provide channels for pharma to access and work with academics.
- Encourage dialogue between academics and industry, discuss university research agendas with companies, and build a strong interface populated with technology transfer and commercialisation professionals.



Slawomir Majewski, Deputy Rector for Science and International Relations, Medical University of Warsaw

Building on academic heritage

As Slawomir Majewski, Deputy Rector for Science and International Relations at the Medical University of Warsaw told delegates, the university has a long history dating back to the 18th century, and today is internationally recognised for aspects of its research. However, in common with the rest of Poland and other newer member states, there is no structure for translational research and commercialisation. One aim of the BASTION project is to put a coherent framework in place.

There are some significant components on which to build, with more than 1,000 students following 17 study programmes and specialists with a focus in cardiovascular disease, oncology, immunotherapy, transplantology and infectious diseases. This scientific potential is to be combined with a €100 million investment in the Centre for Preclinical Research and Technology, (being built in collaboration with two other universities), which is due to open its doors at the start of 2014.

Another key initiative is the formation of the Academic Centre for Innovation, funded by the Polish Government as a foundation stone for improving commercialisation and technology transfer. A first step will be to educate scientists about commercialisation of their research, highlighting the significance of protecting intellectual property and building a culture of systematic knowledge management.

"So we have huge potential, with good labs, good science, and industry is coming: Amgen is talking to us; AstraZeneca is very interested in one of our projects," Majewski said.

However, there are missing pieces in the translational medicine picture, that are now being addressed by BASTION, as Jakub Golab, project coordinator described. These include:

- A low level of international collaboration
- Shortages of human capital, with no positions for regular post-docs (though this is starting to change)
- Bioinformatics
- Lack of equipment

Jakub Golab, BASTION Project Coordinator, Medical University of Warsaw

"So this is why we set up Bastion: to bring together the best research in oncology, get in touch with partners at top universities and the SMEs at the interface, create critical mass and change the face of research at our university"

Jakub Golab, BASTION Project Coordinator, Medical University of Warsaw

- Limited external awareness of the Medical University of Warsaw
- Limited understanding of the importance of intellectual property management, patenting and technology transfer

"So this is why we set up BASTION: to bring together the best research in oncology, get in touch with partners at top universities and the SMEs at the interface, create critical mass and change the face of research at our university," Golab said.

BASTION is making headway in filling the gaps, with a number of academic collaborations now in place, covering a range of research topics, an in-house bioinformatics group has been formed, new assays and animal models are in use.

BASTION is funded from September 2012 to February 2016. However, Golab estimates it will take 5 – 7 years for the full impact of the programme to be pulled through.

Learning from the pathfinders

BASTION may be in its early stages, but the defined work packages and implementation plan highlight how much has been learned from the universities that are the pathfinders in technology transfer, such as KU Leuven, which in May 2013 saw the formation of its 100th spin-off company.

As Olivier Lescroart, IPR Officer at KU Leuven put it, "Universities take their technology transfer role seriously, but we are learning by doing."
In translating basic research through to the clinic, initiatives such as CD3, the Centre for Drug Design and Discovery, PharmAbs, which characterises and validates novel antibodies, and the Clinical Trial Centre, "all came out of problems we encountered," Lescroart said.

Alongside basic research and teaching, universities now view the creation of industry-ready knowledge as one of their fundamental roles. Industry-ready knowledge must have some proprietary aspect to it, must be de-risked in terms of having clear-cut IP and a good safety profile, and must be market-relevant. Universities need to acquire and develop new skills, and develop or get access to new facilities, in order to generate such knowledge.

Apart from expertise in legal aspects of intellectual property, this includes adding a translational research layer, for example the capability to screen for hits against new drug targets or the resources to carry out an

Olivier Lescroart, IPR Officer, KU Leuven

academic clinical trial, Lescroart said.

It also requires seed funds to get start-ups off the ground, and incubator facilities where fledgling companies can find independence from their university roots.

Look for best practice

While spin-offs may be the most visible manifestation of a successful technology transfer office, research collaboration and consultation will form the majority of its activity. This underlines the fact that a critical component in creating a robust technology transfer infrastructure is to have good relationships with local research-intensive companies.

This presents a challenge in translational medicine in Poland, where there are few pharmaceutical companies doing R&D, noted Marcin Szumowski, Business Development Director of the BioTech Med Cluster Mazovia and Head of International Cooperation at the Nencki Institute of Experimental Biology in Warsaw.

The BioTechMed Cluster supports commercialisation of research from scientific institutions across the Ochota Research Campus in Warsaw. As a new initiative, the Cluster is looking to places where technology transfer is more mature, such as Leuven and Oxford, for best practice.

Ask the Entrepreneurs

One of the greatest sources of insights in medical technology transfer comes from scientist-entrepreneurs who have experienced this white knuckle ride at first hand.

Daniela Couto, co-founder and CEO of the Portuguese regenerative medicine specialist Cell2B, described the good and the bad of the uneven environment in which the company is attempting to navigate a path to staging a formal clinical trial of its autologous bone marrow cell therapy for treating organ rejection in transplant patients.

Daniela Couto, co-founder and CEO , Cell2B

On one hand there is a positive regulatory framework in Europe, it has been possible to attract staff with pharma industry expertise in manufacturing and quality control and the University of Lisbon provided an incubator space. On the other hand the company spent much of its existence struggling to find VC investment before persuading three business angels to back it.

“There’s a problem with the limited early-stage venture capital money in Europe,” Couto told the meeting.

Clinical need

The most important aspect of translational medicine is to identify the clinical need, suggested Sabine Bahn, founder of Psynova Neurotech, a company developing blood tests for psychiatric disorders. “There’s a big need in psychiatric disorders, where there has been little change in how they are diagnosed over many decades,” Bahn said.

Bahn, who is also director of the Cambridge Centre for Neuropsychiatric Research at Cambridge University, set up Psynova to commercialise 15 years of basic research in which she had identified distinctive biomarkers in the blood of patients with psychiatric disorders that she believed could form the basis of diagnostic tests.

One important factor in going about forming Psynova was the mentoring of Chris Lowe, Professor of Biotechnology at Cambridge University, who has himself been responsible for getting several start-ups off the ground. Another was receiving initial funding from the Cambridge University Challenge Fund. Despite this support, it was a “slalom course” to get sufficient funding, which was raised as a mixture of venture capital investment, debt finance, and support from a

Sabine Bahn, founder, Psynova Neurotech

commercialisation partner, Rules Based Medicine.

After Rules Based Medicine bought out the VC investors, it too was acquired by Nasdaq-quoted Myriad Genetics. With one test for schizophrenia on the market in the US, Bahn continues to work with Myriad to develop other diagnostics. Having access to Myriad’s resources has made a big difference. “It’s a relief not to have all that weight on my shoulders anymore,” Bahn said.

Tapping pharma’s new-found appetite for early stage research

One of the most significant and positive shifts in the translational medicine landscape in the past year or so, is the pharmaceutical industry’s new-found appetite for early-stage research and its more outward-looking approach to collaborating with universities, biotechs and peer companies.

“We want to build collaboration,” Araz Raoof, Global Functional Head, C.R.E.A.Te, Janssen Research and Development told the workshop. A recent example is the launch of a €5 million collaborative initiative with Belgian academic institutions and research centres to drive discoveries

to improve the prevention, diagnosis and treatment of neurodegenerative diseases.

The project seeks to attract leading researchers in Benelux to submit proposals for research in neurodegenerative disorders. Janssen Research & Development will collaborate initially with KU Leuven, University Hospitals Leuven and VIB (Vlaams Instituut voor Biotechnologie) to create opportunities for collaborative research, and will invite other academic institutions to join the project as its next phase begins later this year.

In addition, Janssen is involved in pre-competitive research being carried out by the European Union's €2 billion Innovative Medicines Initiative (IMI), and Raoof suggested that IMI can form an important element of university technology transfer strategy. Universities need to be proactive in talking about their capabilities. "The opportunities for pharma are large, there are many offers out there and the challenge is to filter them," she said.

Barriers to translational medicine in Europe

Systems for translational medicine in Europe need to accommodate another major shift in the landscape, in the shape of recently-erected post-approval barriers, including health technology assessments, and negotiations with reimbursement agencies and payers that are now putting the focus on value as much as on efficacy.

The conventional view is that the scope of translational medicine is from the lab bench to the clinic, but it is important to get from the clinic to real patients.

Patient access should be factored into the earliest stages of research to ensure the right data is generated for regulatory approval and for payers. "Make sure the regulators are on board, make sure the payers are involved – and at a very early stage," advised Magda Chlebus, Director of Science Policy at the European Federation of Pharmaceutical Industries and Associations.

For Arnd Hoeveler, Head of Unit – Advanced therapies and systems medicine, DG Research and Innovation at the European Commission, another change that needs to be addressed in translational medicine is its increasingly disciplinary nature. Specialists from medical technology, information technology and biotechnology need to work with each other. "Minds have to be opened up and experts brought together," Hoeveler said.

"Learn from other clusters around Europe, this will help in devising strategy"

Arnd Hoeveler, Head of Unit, DG Research and Innovation, European Commission

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Hoeveler also acknowledged that effort is needed at an EU level to create a more robust translational medicine infrastructure. And the traditional 'publish or perish' mandate under which academics operate has to change: there need to be incentives to innovate – which may mean holding back on publication.

Whatever gaps there may be, there is also a lot of expertise in Europe. "Learn from other clusters around Europe, this will help in devising strategy," Hoeveler advised.

Marta Czanik-Kawecka of the Academic Centre for Innovation at the University of Warsaw reflected on some of the practical problems encountered in creating a translational medicine system from the ground up. Critically, there is the question of how to reach scientists and make them interested in commercialisation. There's another challenge in assessing if knowledge is industry ready and also in addressing legal issues, especially in protecting intellectual property.

The Centre for Innovation was created a year ago, and still has some way to go in building an entrepreneurial culture. But the good news is that, "young people are really interested in the opportunity to develop products," Czanik -Kawecka said.

About BASTION

BASTION (From Basic to Translational Research in Oncology) is a multidisciplinary project co-financed by the European Commission under the 7th Framework Programme - REGPOT 2012-2013.

The objective of the project is to build up the research potential of the Medical University of Warsaw (MUW) in the field of experimental oncology.

The scientific research of the highly professional BASTION project teams is focused on personalised medicine and the development of diagnostic and therapeutic methods customised to patients' individual needs.

The project also aims at reducing the time from scientific discovery to clinical application. Therefore much effort is put into increasing innovation within MUW and to support science and business.

More information:
www.bastion.wum.edu.pl/en

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Words: Nuala Moran
Design: Giulia Biasi
Photograph: Carlos Nomen

Roundtable Partners

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