IMS-MRL
Wellcome-MRC Institute of Metabolic Science
- Metabolic Research Laboratories

Translational Research Strategic Plan

CONFIDENTIAL - APRIL 30, 2021
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This Translational Research Strategic Plan has been developed in collaboration together with:
✓ External Consultants with extensive expertise and experience in translational research science and drug discovery & early development in Academia Innovation, Start-up/Biotech and Pharma
✓ Newly-formed internal Translational Research Strategy Group headed by the Director of the Institute, with representative IMS-MRL scientists across fundamental to pre-clinical to clinical research, and the Director of Research Operations (acting as coordinator)
✓ Discussions with many IMS-MRL principal investigators, group leaders and members
✓ In liaison with Cambridge Academy of Therapeutic Sciences and Cambridge Enterprise
INTRODUCTION

Translational Research can be defined as the process of turning discoveries and observations in the laboratory, clinic and community into interventions that improve health and well-being – from diagnostics and therapeutics to medical procedures and behavioural changes. This is optimally developed through interactions between research based in academia, in the health care delivery sector and in industry.

The IMS-MRL research approach “From Bedside to Bench and Back to Bedside” on the aetiology and pathophysiology of human metabolic and endocrine diseases is bringing critical information towards understanding the underlying molecular basis of these diseases, towards improvement of diagnosis, therapy and prevention of some rare extreme phenotypes, and critical insights for treatment of highly common metabolic diseases. The IMS-MRL overall strategy is to highlight and further develop its translational science aiming to make a major contribution to pharmacological, nutritional and behavioural interventions to benefit not only patients with rare metabolic or endocrine disorders with high unmet medical needs, but also to the metabolic health of the general population, tackling the major and global health concern of obesity which causes considerable ill health with severe associated problems such as diabetes, cardiovascular disease, disorders of the gastrointestinal tract, and more recently to significant increased risk of death by Covid-19 infection.

Many key criteria required for successful translational research are already present at the IMS-MRL and its surrounding environment:

✓ Targeting diseases with high unmet medical needs
✓ Patient-centric approach
✓ Strong scientific heritage
✓ Interdisciplinary research
✓ Key collaborations with academics and biotech/pharma
✓ Established Translational Research support within University
✓ Fertile “fostering innovation” surrounding environment around University

Translational Research at IMS-MRL already covers a wide range of activities, including:

✓ Development of diagnostics and therapeutics based on the discovery science in the Institute
✓ Development of specialist clinical services in the NHS
✓ Consultancy – providing expert advice to industry
✓ Fee-for-service contract research
✓ Collaboration with industry in fundamental discovery research and in industry-led translational research e.g. Phase 2 and 3 clinical trials
✓ Provision of policy advice to government

The overall objective of this plan is to further develop a competitive Translational Research Strategy at IMS-MRL and establish an efficient operating process/pathway to support translational research activities and generate new opportunities in discovery research, with the outcome of resourceful new knowledge relating to human metabolic health, metabolic disease and its effective treatment, which will have the potential to significantly improve public health and wellbeing.
STRATEGIC GOALS & OBJECTIVES

The objectives listed under each strategic goal are to guide IMS-MRL in further developing, evaluating and refining its translational research over time, with some example approaches of ways to achieve these objectives, with the overall aim to grow a strong translational science arm.

Strategic Goal 1: Articulate On-going Translational Research Activities

Objective 1-1: Optimising visibility of on-going translational research activities

This could be achieved by creating a dedicated “Translational Research” webpage section on the IMS-MRL website as a non-confidential overview of the translational research tools, approaches and activities of the entire institute – creating in addition weblinks to each related core technologies/groups/metabolic unit sections with the following contents:

- **Highlighting translational research skills and competencies with clinical, preclinical and fundamental research tools and technologies.** Some representative examples could be briefly described in the key cores of:
  - Translational Research Facilities (TRF) - NB: The existing TRF web-section in the “Core Facilities” section of IMS-MRL website could be linked to the potential new “Translational Research Activities” web-section, with a brief description of some projects illustrating the translational science
  - Clinical research diagnostic tools
  - Preclinical research “tool-box”: *in vitro & in vivo* models already developed and successfully utilised in preclinical translational research or earlier steps of translational research
  - Basic research “tools and technologies”: e.g. development of cells, antibodies, assays, genetic tools/models, which can empower high-quality and innovative translational research

- **Highlighting past and current translational research related projects.** An overview of key translational research therapeutic areas and activities could be formulated, with also some representative examples throughout, including in:
  - Genetics and clinical translation for rare diseases in endocrinology and metabolism
  - Targeting the integration mechanisms of neural circuits controlling appetite, satiety, body weight and blood glucose
  - Regulation of the gut-brain axis – Effects of bariatric surgery
  - Therapeutic and interventional strategies for thyroid disorders, obesity and diabetes

Together with representative examples of:
  - Clinical research “tool-box”, development of diagnostic tools, development of specialist clinical services in the NHS
  - Preclinical translational research with state-of-the-art cell-based functional assays and predictive pharmacological and disease models
  - Fundamental scientific research, including cutting-edge human genetics and cell models

All contributing towards translational advances e.g. identification/validation of targets, mechanisms of biological and pharmacological actions, diagnostic tools, and early clinical development (including with human cell models), or therapeutic interventions.

IMS-MRL Translational Research areas, approaches and activities overview and representative key examples are briefly described in the different Appendices A1.
Objective 1-2: Formulating the on-going translational research “discovery cascade”

- Highlighting the already on-going steps of the research "discovery cascade - From Bedside to Bench and Back to Bedside", which could be presented in the future specific “Translational Research” section of the IMS-MRL website, in key areas such as:
  - Human proof-of-concept with genetic studies / Basic science research & target identification
  - Cutting-edge assays and technologies development for mechanism-of-action studies and for potential translation to target validation and lead optimisation in relevant functional rodent and human cell-based models and assays
  - Pharmacological disease models for preclinical translation (e.g. target validation)
  - Biomarker development (e.g. for early diagnostics)
  - Interactive “loop” between preclinical and clinical research
  - Clinical development phases

A conceptualisation of the “discovery cascade” of Translation Research at IMS-MRL in the context of the Drug Discovery Research & Development cascade is schematised in Appendix A1.4.

A potential useful approach could be to pinpoint (internally) all on-going or at the idea-stage translational activities at the different steps of the cascade to help identifying some gaps that could be interesting and pertinent to address and develop as new translational opportunities.

Objective 1-3: Encouraging flow of information on translational research activities

- Encourage all researchers already involved with translational research activities to add their input in the IMS-MRL website inside their group/PI section and/or core technology section. A shorter-term objective, which should be readily achievable, would be to engage each PI/ Early Career Researcher/Core Facility Director in adding to their related IMS-MRL group webpage a brief overview paragraph on their on-going or planned, or potentially leading to, translational activities entitled e.g. “Translational Research” or “Therapeutic Translation” (as some have already done), “Transformative Relevance to Health and Disease”, “Translational Research Approach”, “Relevance of Preclinical Research to Humans with Disease”, “Potential Translation of Biological Discovery”, mentioning also external collaborations and particularly with biotech/pharma. (NB: separately, it could be relevant to discuss a potential “intranet” to share more detailed info within the Institute regarding e.g. translational research tools and models available, also a very useful “repository” especially for new comers).

This could be also an immediate step toward increasing visibility, while waiting for resources needed to create a specific website section on “Translational Research”.

It could be also valuable that in each group and/or core technology section, an update of the publications is regularly done, and also a weblink is attached to the names of each Post-doc or PhD student to provide a short synopsis CV – some are already using a weblink to the Cambridge Metabolic Network; some PIs have also developed in parallel some separate group websites including a short biography of every group member.

- Engage with all researchers at the institute through informal interactions focused on translational science, through for example some informal presentations of on-going translational activities in regular internal “pitching” mini-symposia and/or reserving some time during lab- or dept-meetings to highlight some of these on-going activities and ideas.
• Engage preliminary contact at any time with Cambridge Academy of Therapeutic Sciences (CATS): for some guiding information, on translational research project(s), even at just the idea stage; for some education & training support; for information on funding opportunities; or who/when/how to interact with other Translational Research supporting bodies at Cambridge. (see brief overview on CATS and Cambridge Enterprise in Appendix 2)

• Engage preliminary contact with Cambridge Enterprise, particularly on Intellectual Property initial assessment, and quite early in the project to avoid any potential issues.

Strategic Goal 2: Advancing Translational Science

Translational science can be defined as a multi-directional and cross-disciplinary process of scientific research committed to patients, and encompasses the different stages from fundamental and pre-clinical research and discoveries made on the biological basis of health and disease, to clinical research and clinical implementation of interventions that can improve the health of individuals and the public – from diagnostics and therapeutics to medical interventions and behavioural changes.

IMS-MRL is committed to develop further its translational research activities across the full spectrum of translational science with the focus on unmet needs of patients with critical metabolic disorders, as well as to contribute to “cultivating” the next generation of translational scientists.

Objective 2-1: Developing the translational “expertise” through an engagement strategy

A primary goal is to stimulate and help IMS-MRL scientists at all levels and fields of research develop better understanding of the still emerging field of translational research in academia and get engaged in it, firstly by exploring the translational perspective of some of their research activities and its potential impact on the discovery research process.

A complementary key goal is to propose some support training and to facilitate interactions with consultants expert in the key stages of disease drug discovery and clinical development.

• Translational Research groups/bodies to support, advise, facilitate and catalyse translational research activities:
  - IMS-MRL “Internal” Translational Research Strategy Group i.e. small group of internal researchers as reference contacts, advisors, facilitators to implement the strategy. An initial group of representative IMS-MRL scientists across fundamental to pre-clinical to clinical research with the IMS-MRL Director and the Director of Research Operations (as coordinator) has been already set-up to discuss and develop the translational research strategy together with external consultants.
  - IMS-MRL “External” Translational Research Advisory Committee/Board e.g. to build an initial small group of external consultants with extensive experience in collaborative Academia/Industry translational research projects and discovery research & development, acting as a sounding board of mentors/advisors/catalysts on a voluntary basis, to give confidential, impartial and constructive feed-back advice, for example one day/month.
• **University of Cambridge Support Services for Translational Research:** (see brief overview of Cambridge University bodies supporting translational research in Appendix A2)

A comprehensive “Map” of this already existing network would facilitate interactions with relevant experts, including industry and/or technologies experts, intellectual property experts and regulatory experts. CATS is currently coordinating and preparing such a “map” as an overview guide-line for researchers on how/when/who to interact with for any questions regarding translational research ideas and projects at different stages of development.

- **Education & Training related to Translational Research:**

Training of clinical and non-clinical scientists is a major ongoing activity of the Institute, with many past trainees now working in industry. In addition, further training could be set-up e.g.:

- **Highlighting various easily-accessible sources of information and (free) events related to translational research**, such as publications in Science Translation Medicine, Drug Target Review, Current Trends in Drug Discovery, Expert Opinion on Investigational Drugs, and free webinars and events e.g. from Science journal website, BIA UK Bioindustry Association, the European Laboratory Research & Innovation Group ELRIG UK (e.g. online June 2021 event on “Translating Ideas into Therapies” co-hosted with the British Pharmacological Society).

Concrete examples of translational research achievements can also be found in publicly available documents e.g. “Expanding horizons: Realising the potential of MRC science” prepared by LifeArc, Translational Research charity organisation, which also looks after the MRC’s intellectual property and technology transfer needs.

Glancing also through (free) online sources of professional business information could help familiarising with the “industry” approaches e.g. with Start-up/Biotech/Pharma/Health Care Industry news in BioPharma Dive, and/or Fierce Biotech, and/or BIO Smart Brief, and DiabetesPro SmartBrief, or attending (free) webinars e.g. from J&J Innovation/JLABS.

More specifically for junior scientists, highlighting forums for post-docs, e.g. for “Pitching ideas“ forums or “Postdoc Business Plan” competitions at Cambridge University, or following local news about entrepreneurial science and technology start-ups (e.g. in business section of “The Cambridge Independent”) or events such as the Biochemical Society “Industry and Academic Collaboration” awards. Also attending drug discovery related (free) webinars or workshops organised by some biotech/pharma (e.g. Metabolon, Charles River Laboratories).

- **Organising for Fundamental and Pre-clinical researchers a set of seminars by discovery research experts illustrating translational science through concrete examples**, with the support of the different Cambridge University bodies, such as CATS, Cambridge Enterprise, the Milner Therapeutics Institute, the Office for Translational Research, and taking opportunity of their access to industry consultants, entrepreneurs-in-residence and successful local spin-out/biotech translational scientists.

In addition, some “tailored” seminars could be organised internally by inviting local and national “entrepreneurs” who have developed their translational science into start-ups/biotechs, and therefore with a clear and pragmatic view of how to develop translational activities (e.g. scientists from Cambridge-based Astex Pharmaceuticals or Stemnovate Ltd).

- **Support training for Clinician-scientists** by developing further the on-going internal clinical training, also organised through the Translational Research Facility core, aiming to act as a
knowledge hub to support the training of the next generation of clinician scientists, locally, nationally and internationally, including in ethical and regulatory submissions. Some additional useful sources of information could be found for example on the European Medicine Agency website (e.g. guideline on strategies to identify and mitigate risks for first-in-human and early clinical trials with investigational medicinal products).

- **Highlighting “Entrepreneurship” training opportunities**, e.g. with Cambridge Enterprise hosting several “commercialisation” surgeries and webinars (including on Intellectual Property). “Accelerator Business Programmes” are proposed by several organisations including StartCodon - the Cambridge Health Care Accelerator, One Nucleus Cambridge-based not-for-profit Life Science & Healthcare organisation, BioCity/WAPG, largest scientific incubator in the UK, which could guide scientific “entrepreneurs” to develop their discovery science plans, launch and grow businesses from their translational activities.

For more junior scientists, on-line courses empowering researchers to innovate are also available e.g. from “Postdocs to Innovators” p2i consortium (including the University of Cambridge) with flexible self-directed courses such as “Develop your entrepreneurial mindset and enterprise skills: seizing opportunities and seeing them through”, or courses proposed to students by i-Teams Cambridge (on Innovation, Development or Medical) which combines multi-disciplinary teams of students with industry mentors and real University inventions to assess the commercial viability of new technologies and product designs (see Appendix A2).

**Objective 2-2: Fostering innovative discovery science and multi-disciplinary partnerships**

To develop translational research activities with a focus on long-term sustainability, it is crucial to establish strong support through easy access to a network of relevant experts and to multi-disciplinary collaborations. At all stages, it is essential to develop innovative approaches and validate their usefulness, and to share discoveries.

This is therefore vital to “connect right from the start” with:

- Identifying “gaps” to optimise coordinated commitment from clinicians and basic and applied researchers, within the different research groups and technology cores
- Understanding target profile and patient population early in the development process
- Maximising feedback loop information between pre-clinical and clinical scientists
- Build-up of mature cooperation of skills and capabilities essential to address the multidisciplinary requirement and high cost of discovery research
- Understanding how to establish constructive partnerships between academic basic disease research and industry translational science
- Exploring possibilities of funding through academia-industry cooperation that could include some guiding from the start, e.g. with Knowledge Transfer Partnerships (KTPs) scheme from Innovate UK (now part of UK Research and Innovation)

**Innovative translational research could span the full spectrum of translational science:**

- **Clinical translational science innovation**

Many key factors required for the development of cutting-edge clinical translational science are already present at IMS-MRL, which is a centre of excellence and international reference for
studying human extreme phenotypes of metabolic disorders, and bringing significant insights towards better understanding of metabolic health and disease. Key components:

- Clinical trials and directly related mechanism of action studies
- Clinical research “tool-box”
- Rare diseases clinical research international network
- Engagement with patients and with community groups
- Dedicated clinical data and patient registries
- Driving multi-disciplinary collaborations with academic and clinical partners
- Engagement with private-sector partners (including with angel investors or venture capital firms)
- Focus on supporting the collection of long-term scientific findings while enabling disseminating data rapidly, thus enabling monitoring of data by researchers and bringing new research insights to metabolic diseases within the general population

The clinical research innovation at IMS-MRL has also been fostered by the development of specialised websites and portals, complementing the general IMS-MRL website (for example, the websites www.goos.org.uk and www.stilts.org.uk implemented and coordinated by Professor Farooqi, and specifically directed towards translational activities related to the genetics of obesity and thinness, are highly-collaborative points of international reference). Moreover, the need of establishing other similar websites for other rare clinical disorders studied at IMS-MRL (e.g. some thyroid hormone disorders), and several highly developed portals allowing collection of largescale data over extended periods, has already been identified. It would therefore be important to explore the possibilities for some external funding from the support functions at Cambridge University to get some specialist help with the setting up and maintenance of such relevant portals as key clinical translation research tools.

Other key components required for the development of innovative translational science at the stages of pre-clinical and fundamental research are also present at IMS-MRL, supported by multidisciplinary and cutting-edge technology cores in a highly collaborative environment. Some basic research in academia can be run similarly to exploratory discovery research (that could also be defined as basic research) in industry, and can thus be defined as early translational research, as it may lead to translational advances such as new physiologically relevant cellular tools, pharmacological models, targets or mechanism-of-action insights.

- **Pre-Clinical translational science innovation**
  - Early proof-of-concept with gene-edited pharmacological models
  - Relevant and complementary state-of-the-art disease-related rodent models
  - Strong interactions between pre-clinical and clinical research
  - Full range of experimental capabilities for metabolic research

- **Fundamental research and translational science innovation**
  - Early proof-of-concept with human genetics discoveries
  - Relevant rodent cell-based tools and functional assays
  - Cutting-edge human cell models
  - Understanding normal biological and pathophysiological mechanisms
To continuously improve translational effectiveness and to accelerate the early stages of ideas, it might be essential to:

- Identify the interventional gaps within the “discovery cascade” and to bridge these gaps by collaborating with discovery scientists in research areas such as medicinal chemistry, pharmacokinetics, toxicology
- Foster complementary connections and sharing of data and knowledge, including with other academic or biotech/pharma scientists, for example by accessing tools such as benchmark/lead compound/biologics (with some freedom-to-operate)
- Design and implement a process for measurable results

**Objective 2-3: Identifying new opportunities of translational research**

Unlocking the full value of IMS-MRL research into translational advances could be built upon its already on-going translational activities, and be envisaged for example through expansion of:

→ generation of intellectual property from research discoveries (e.g. novel reagent or assay, novel utility of existing agent, novel diagnostic tool, novel potential therapeutic agent)
→ generation and provision of services for patients (e.g. diagnostics or therapeutic interventions, services for NHS)
→ provision of services to industry (such as consultancies, fee-for-services e.g. testing of potential novel therapeutic agents for metabolic diseases in humans and specialised models)
→ collaboration with industry (e.g. to explore novel potential drug target, to investigate underlying mechanism(s) of target-specific or unknown-target agents, to develop new tools and models)
→ contribution to scientific and to public policies (e.g. advice to government or other bodies on nutritional strategies to combat widespread obesity, and promote awareness of rare diseases)

Several approaches could be explored to identify new opportunities of translational science, e.g.:

- Structuring collaborative translational research project proposals attractive to potential “industry” partners who can benefit from the unique disease areas of expertise at IMS-MRL, and can provide drug development experience and capabilities, and with whom to share some common translational research objectives such as:
  - Identification of drug targets or disease phenotypes
  - Assay/model development for mechanism of action studies
  - Testing activity of target-specific compounds/biologics in cellular assays and *in vivo* models
  - Advancing knowledge in disease mechanisms of action
  - Identification of active compounds/biologics that could reduce disease phenotypes
  with the overall goal of accelerating target identification and validation and to advance newly identified promising candidate therapeutics through late-stage preclinical development, towards an Investigational New Drug (IND) application for clinical testing.

- Articulating to potential “industry” partners how research findings on the molecular basis of rare and neglected diseases can be translated into effective medical treatment for these extreme metabolic disorders and can also lead to opportunities within larger populations with metabolic disorders, and can furthermore provide unique opportunities to advance the field of therapeutic development and technological innovations at crucial stages.
• Assessing drug repurposing strategies (e.g. early-stage repurposing to identify therapeutic leads, and late-stage repurposing to prepare therapeutic leads/candidate for clinical testing), which can reduce time-frame, costs and risks of drug development, and improve success rate.

• Exploring opportunities such as getting access to:
  - relevant “drug discovery” tools such as prototype compounds of new or validated targets (including through publicised industry collaboration proposals, e.g. AstraZeneca Open Innovation scheme, or partner & open innovation and compound sharing opportunities with Novo Nordisk)
  - collaborative academia-industry funding e.g. through Innovate UK (and more particularly the KTPs scheme which links forward-thinking businesses with the UK’s world class knowledge bases to deliver innovation, accompanied by a KTP associate to manage the collaborative project) or e.g. through LifeArc (with also a step-wise approach from getting in touch to submit an idea, to completing a project outline form in collaboration with LifeArc “targets team”, to a two-stage review process, before commencing a collaborative feasibility work – NB: each MRC unit and Institute has also a dedicated LifeArc business manager who will work with researchers to identify inventions with commercial potential.)

• Exploring opportunities to maximise value of development of cutting-edge in vitro and in vivo models and assays through collaboration with and/or licensing to established Contract Research Organisations.

• Monitoring world-wide drug discovery projects at different stages of development in start-up, biotech and pharma to identify collaborative opportunities of complementary expertise.

**Strategic Goal 3: Developing Operational Model and Process/Pathway**

Developing an effective operational model facilitating the process of translational and discovery research should be accompanied by an awareness of the importance of getting housekeeping issues right, of strengthening the conversation on strategy and long-term performance, and of providing feedback in a way that adds value to all participants.

**Objective 3-1: Developing guide-line process/pathway to support translational research**

It is crucial to develop some guide-lines for the different stages of translational research: from using original basic or clinical scientific research to generate an idea of a new target, technology, diagnostic test or treatment, to some initial proof-of-concept, to challenge/discuss it with the different support groups and experts in discovery research, to then propose a full development plan highlighting the novelty and competitiveness of the project’s purpose, with potential patient benefit and possible partnerships. Several approaches such as:

- Developing step-by-step “map” including who/when/how to involve different support groups and potential collaborators (e.g. collaboration with industry partners providing drug compound/biologic starting point and IMS-MRL providing disease expertise)
- Proposing template(s) for translational research project proposals with preliminary outline plan, including draft of step-wise strategy for the project development and decision criteria
- Providing project management and drug development advisory support to advance the project (including key scientific milestones and time-lines)

Some suggested translational research / discovery project proposal templates, also useful to prepare “communication” documents for exploring and/or developing potential collaborations or get appropriate feed-back from relevant advisors, are outlined in the Appendices A3.

In addition, some examples of specific requested templates can be found on different relevant websites such as “Translation Award Concept Note” from Wellcome Trust, a “Disclosure Form – Idea and Innovation” from Cambridge Enterprise, “Target Innovation Research Proposal” or “Preclinical Research Proposal” from OpenInnovation AstraZeneca, or LifeArc “non-confidential idea” submission form.

Objective 3-2: Awareness of specific procedures required for translational research

Committing to translational research in academia, which can be perceived as a complementary and more directed pathway to impact research for human health, requires some shift of mindset and also some implementation of discovery research procedures in areas such as:
- Recording of data
- Contingency plans regarding critical tools and research materials
- Generation of preclinical and clinical data discovery-quality material
- Carefully-thought publication strategy to secure freedom-to-operate and/or protection of intellectual property (IP)
- Project management skills; Development of engagement strategy with collaborators

These procedures, that could initially be felt as constraints, should be relatively straightforward to implement and would provide an essential security to safe-guard translational research projects at their very start, allowing then the potential to develop them fully as discovery projects in pharmaceutical partnerships or to attract a licensing partner or to explore the possibility of spinning-out towards start-up creation.

Awareness of time and resources required for specific processes such as patent application (e.g. need to evaluate potential outcome value of IP as compared to high costs inherent to application and maintenance) is also essential. Early contact with supporting bodies is recommended.

Objective 3-3: Continuous assessment for competitive translational research

Developing competitive translational and discovery research projects should require some regular monitoring and continuous assessment of key criteria such as:
- Added value and “staying at the cutting-edge”
- Competitor status
- Potential to move forward into human trials and transform patient care
- Potential interest from industry-based translational science
- Potential “business” development plan (with support from CATS and Cambridge Enterprise)
This should also help identify priorities and challenges in key areas of translational research, especially if/when unexpected data or new information regarding the project area arises. Flexibility and need to react promptly are also key factors to successfully progress such projects.

**RESOURCES REQUIRED TO IMPLEMENT TRANSLATIONAL RESEARCH STRATEGY**

The speed of vaccine development during the Covid-19 pandemic has clearly demonstrated the almost incalculable value of a strong academic research and wider scientific base for the discovery and development of successful and indispensable new therapeutics and also showed how the vast resources and capabilities of biotech/pharma companies can bring this to a reality.

Fostering the recognition and growth of translational science as well as cultivating the next generation of translational scientists, and catalysing innovative partnerships to advance discovery research, require extensive support, with training opportunities, and training resources and tools, and also powerful sources of information tools, including a dedicated extensive core section in the IMS-MRL website, and dedicated portals for clinical research.

Therefore, there is an absolute need of some extra funding dedicated to implement this strategy. Some draft budget proposal about projected costs for website editing and portals expansion, and any potential additional substantial costs (e.g. for a dedicated IMS-MRL coordinator / operational manager for Translational Research) – could be prepared internally in order to be able to apply for some funding with the help and guidance of CATS.

In addition, it may be also relevant to explore the possibilities of different “business” models from fee-for-service to collaborative industrial funding, as developing translational research activities may require substantial additional resources as compared to academic basic research.

**CONCLUSIONS & SWOT ANALYSIS**

The distinctive “From Bedside to Bench and Back to Bedside” approach of IMS-MRL, combined with its “Treasuring the Exceptions” approach to study the genetics and mechanisms underlying rare metabolic phenotypes, and together with its cutting-edge research into the regulation of energy homeostasis and molecular and cellular mechanisms underlying metabolic disorders, delivers critical insights into metabolic chronic diseases such as obesity and diabetes across the wider population, and gives the Institute a world-wide competitive edge to further develop translational research activities towards addressing critical unmet medical needs.

Most therapies today focus on alleviating symptoms rather than addressing the causes. Translational activities at IMS-MRL, with a unique combined expertise in some key *in vitro* models, pharmacological *in vivo* models and clinical set-ups, and focusing on critical biological pathways shared across several diseases, could help identify and develop novel and more precisely targeted treatments.

A well-defined translational research strategy at IMS-MRL should allow to highlight more visibly the on-going translational activities, to help identify new opportunities with a clear path on how to develop them, and to help maximise the value and impact of IMS-MRL research output.
An initial analysis of the strengths, weaknesses, opportunities and threats (SWOT analysis) of this engagement strategy should help highlighting the opportunities to progress and the hurdles to overcome, and is summarised below.

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<tr>
<th>SWOT Analysis</th>
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<tr>
<td><strong>Strengths</strong></td>
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<tr>
<td>• Unique expertise and commitment to rare metabolic disorders with the strong philosophy that science and compassion must work together to make meaningful differences for patients and communities in need.</td>
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<td>• Fore-front expertise in human genetics, and know-how to translate findings into <em>in vitro</em> and <em>in vivo</em> disease models to decipher critical biological pathways.</td>
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<td>• Large body of cross-disciplinary researchers, including many Key Opinions Leaders (KOLs), focused on metabolic disorders, and with cutting-edge technologies and tools.</td>
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<td>• Understanding of scientific and operational principles underlying the translational process, and valuable international scientific and business network.</td>
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<td>• Track-record of successful translational research activities, particularly in translational pre-clinical and clinical science, with well-established Translational Research Facilities (TRF) core and other key core technologies facilities.</td>
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<td><strong>Weaknesses</strong></td>
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<tr>
<td>• Some misconception among academic scientists in general about the specific approaches with clear “measurables” required to develop R&amp;D projects.</td>
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<td>• Bridging the gap between Academia and Industry requires awareness of using common “language”.</td>
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<tr>
<td>• Need to articulate translational research activities or ideas (e.g. with a structured outline of the project with a “roadmap” of scientific and operational standpoints), tailored to the desired audience, in order to stimulate more constructive discussions with “industry” experts and applied research scientists.</td>
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<td>• Need to implement a more systematic approach to further develop academia/industry collaborations: by increasing visibility of translational research activities both internally and externally, and by keeping up-to-date with drug discovery and development relevant projects at biotechs and phamas and “scouting-out” some potential new constructive collaborations.</td>
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<td><strong>Opportunities</strong></td>
</tr>
<tr>
<td>• Enhancing clinical translation with several highly developed portals allowing collection of largescale data over extended periods.</td>
</tr>
<tr>
<td>• Developing translational science from fundamental research findings to identify novel targets or to develop critical screening or mechanism-of-action models, or also to participate to the on-going discovery activities at biotech/pharma, sharing complementary expertise and tools. Maximising potential of unique preclinical models.</td>
</tr>
<tr>
<td>• Developing further biotech/pharma network, and utilising further the rich Cambridge micro-environment of Translational Research supporting bodies and innovative partnerships.</td>
</tr>
<tr>
<td>• Developing further skills (especially for young scientists) towards a wider research “horizon”.</td>
</tr>
<tr>
<td>• Stimulating opportunities (especially for KOLs) to participate to Scientific Advisory Boards of Biotech/Pharma.</td>
</tr>
<tr>
<td><strong>Threats</strong></td>
</tr>
<tr>
<td>• Need to clarify what is the desired balance between fundamental research and translational research at IMS-MRL (and also at the University of Cambridge and with other funding bodies in general).</td>
</tr>
<tr>
<td>• Uncertainty of the future due to the Covid-19 pandemic, and to the lack of funding stability.</td>
</tr>
<tr>
<td>• If goal is to grow translational research activities towards a fully-developed translational research “arm” at the Institute, need time and additional funding, and need to “engage” a critical mass of researchers interested in identifying and/or developing further translational research activities.</td>
</tr>
<tr>
<td>• Need regular assessment of &quot;competitiveness&quot; or “attractiveness”, with flexibility and sense of urgency to re-evaluate project development.</td>
</tr>
</tbody>
</table>
APPENDICES

Appendices A1. Translational Research at IMS-MRL

A1.1. Overview of Wellcome-MRC IMS Translational Research Facilities (TRF)
A1.2. Spectrum of Translational Research activities, approaches & tools at IMS-MRL
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Appendices A3. Templates for Translational Science Project Proposals

A3.1. Preliminary Project Proposal “Road-Map” (for internal use)
A3.2. Non-confidential brief Project Proposal Outline (to engage with translational research supporting bodies or with potential external partners)
APPENDICES A1. Translational Research at IMS-MRL

A1.1. Overview of Wellcome-MRC IMS Translational Research Facilities

The Translational Research Facilities (TRF) at IMS-MRL is a core supporting delivery of internationally-leading experimental medicine and translational research in Human Metabolism and the diagnosis and treatment of Metabolic Diseases (obesity, type 2 diabetes, lipodystrophy, insulin resistance, fatty liver disease, and thyroid disorders). Scientific Director: Professor Sadaf Farooqi

www.mrl.ims.cam.ac.uk/research/core-facilities/wellcome-mrc-ims-translational-research-facility-trf/

TRF Vision and Goals:

→ To accelerate the translation of discovery science, with state-of-the-art environment customised for metabolic research, embedded technologies and highly skilled core staff.
→ To act as a knowledge hub to support the training of the next generation of clinical scientists, by consolidating local skills and knowledge and disseminating nationally and internationally.

Key Assets:

→ Metabolic Clinical Research Facilities custom-built and well-equipped to perform experimental medicine studies on behavioural and nutritional interventions, and clinical trials with potential novel therapeutics. Facilities providing key equipment and methods for critical measurements such as body composition and fat distribution, energy intake and eating behaviour, energy expenditure and substrate utilisation, organ-specific complications of metabolic diseases.
→ TRF Units ideally located on two floors (Eating Behaviour Unit and Metabolic Unit) adjacent to the IMS-MRL Institute and the Wellcome Trust-NIHR Clinical Research Facility, and within the Addenbrooke’s hospital site, thereby facilitating share of critical equipment.
→ Education & Training of clinical researchers on metabolic health, metabolic diseases and its treatment, (including on preparation of documents for clinical trials, experimental medicine studies and ethical approvals).

Key Collaborations and Partnerships:

→ Active collaborations with many IMS-MRL researchers, and also key partnerships locally, nationally, and internationally, including with several Biotechs & Pharmas.
→ Initiation and development of multi-disciplinary collaborative translational research projects involving:
  • IMS-MRL researchers with expertise on rare genetic disorders, neural activity “biomarker”, macronutrient preference and reward
  • Population Science researchers at the University of Cambridge with expertise on metabolic traits, common obesity and diabetes (from MRC Epidemiology Unit), and on food choices and modelling of real-world setting (from Behaviour and Health Research Unit)
  • Industry scientists with expertise in clinical trials & mechanism-of-action studies (from e.g. Rhythm, Aegerion/Amryt, Akcea, Regeneron, Ipsen, GSK, Novo Nordisk, MedImmune/AstraZeneca), and nutrient absorption and eating behaviour (from e.g. NIZO, Biokier, Unilever, Kallyope)

Track-record of significant translational clinical research impact/outcomes:

- Clinical trials at TRF with key impact e.g. to recent approval of setmelanotide by FDA for chronic weight management in patients deficient in key proteins involved in brain satiety pathways.
- Experimental medicine studies with key outcomes e.g. that have defined the phenotype of disorders of thyroid hormone action leading to the diagnosis and treatment of patients worldwide
- Development of a strong international Network of Expertise

Future Development:

→ Further development of technologies, including novel tools and platforms, creation of a Knowledge Repository portal, development of Standards, in order to facilitate new multi-disciplinary collaborations
→ Explore with CATS about new and diverse opportunities of funding and industry collaboration
→ Growing “organically” to assist further translational activities and advances at IMS-MRL, including from emerging discoveries from preclinical and fundamental science
APPENDICES A1. Translational Research at IMS-MRL

A1.2. Spectrum of Translational Research Activities, Approaches & Tools at IMS-MRL

- 2 pages -

For the preparation of this Appendix on Translational Research activities ongoing and/or planned throughout the whole Institute, templates of one-page overview for all IMS-MRL Research Groups were prepared and discussed individually with each Group Leader, with the following key points addressed:

- Main translational research area(s)
- Example(s) of translational approaches, techniques, tools and/or in vitro & in vivo models
- Example(s) of translational research activities in clinical and/or preclinical and/or fundamental discovery science, and/or potential translational application(s) of research
- Example(s) of internal translational research collaborations within the institute
- Example(s) of external translational research interactions/collaborations (including with industry)
- Example(s) of potential future "entrepreneurial" development

A collective overview of some key representative examples selected from these IMS-MRL Research Group templates is presented below (non-confidential information only).

IMS-MRL’s rich spectrum of translational research activities, approaches and tools is the result of prominent interdisciplinary internal and external collaborations and partnerships (including with several biotechs and major pharmas), with critical assets such as:

- Development and access to unique cohorts of patients; stratified medicine
- Human genetics discovery with population genetics (UK Biobank) embedded in leading MRC epidemiology group
- Maximising value of human genetics data by rapidly initiating functional biology studies
- Metabolic and appetite research together in only one centre, with unique set of clinical research facilities with set-up for hospitalised patients
- Dedicated translational research facilities for clinical trials and experimental medicine studies
- Expertise in both central and peripheral metabolism and regulation mechanisms
- Cutting-edge set of human cell models and assays
- Multidisciplinary approaches to murine metabolic phenotyping

Collective overview of key representative examples of the IMS-MRL “translational pipeline”:

- Identification and development of genetic and other biomarkers for metabolic diseases, their introduction into national and international clinical screening services, and acting as a key centre of referral and investigation of rare metabolic conditions: e.g.
  - Screening for congenital thyroid disorders and identifying new variants in neonates and developing new treatment approaches for children with rare variants
  - Severe insulin resistance syndromes (e.g., due to lipodystrophies or insulin receptor variants)
  - Large scale population studies to identify novel genetic and other biomarkers markers for type 1 and type 2 diabetes and susceptibility to these metabolic disorders

- Construction of genetic resources from defined human phenotypic descriptions of metabolic disorders as a means of identifying causative genetic variants; testing and validation of such variants in cell and animal models leading to potential novel drug target validation: e.g.
  - Description of the different mechanisms underlying the abnormal physiology of different human MC4R variants in human obesity
  - Development of and access to a unique group of patients with severe obesity
  - Identification of novel genetic variants leading to excessive thinness
  - Contributions to the National gene panel for Genomics England
(A1.2. cont.)

- Clinical trials with accompanying detailed and comprehensive and relevant metabolic assessment potential (including calorimetry, MRI, whole brain imaging, naturalised feeding, IVGTTs, clamp studies, hunger/motivational measurements) for the testing of novel potential agents for treating metabolic disorders: e.g.
  - Trials involving setmelanotide for obesity and Triac for hypothyroidism, and for novel immunotherapies for individuals with a predisposition for developing type 1 diabetes
  - Treatment and advisory centre for individuals with leptin deficiency
  - Development of and treatment with targeted therapies for patients with lipodystrophies

- Fundamental and preclinical studies on processes, mechanisms and pathways controlling energy intake, expenditure and storage in humans, and their defects causing metabolic disorders, leading to insights into how these can be addressed by nutritional, pharmacological, behavioural and environmental means: e.g.
  - Specialised rodent models for hypoglycaemia and free-moving mouse clamp studies
  - Studies leading to novel dietary and pharmacological interventional approaches during pregnancy to help patients with a predisposition to gestational diabetes
  - Systems Biology approaches to identify organ specific networks relevant to insulin resistance and metabolic diseases
  - GDF15 agonists/antagonists as potential therapies for obesity, cachexia, hyperemesis gravidarum, rare mitochondrial diseases

- Collaborative technical and clinical efforts on novel approaches and technologies to improve blood glucose control and reduce risk of hypoglycaemia in diabetic patients: e.g.
  - Development of Apps controlling closed-loop blood glucose monitoring and insulin pump systems to design an “artificial pancreas” for type 1 diabetics and type 2 diabetic inpatients
  - Expansion of technology of Apps to different glucose monitoring and insulin pump systems
  - Advice to the Secretary of State for Transport on driving and diabetes

- Specialised and collaborative expertise for investigating and mapping brain micro-circuitry and other pathways and molecular mechanisms controlling appetite and satiety and their effects on body weight, using state-of-the-art approaches and techniques such as single-cell transcriptomics, quantitative proteomics, metabolomics and lipidomics; high content and live-cell imaging; cellular co-cultures and organoids; human pluripotent stem cells differentiated into hypothalamic neurones, adipocytes, myocytes, hepatocytes, and intestinal organoid systems; CRISPR/Cas-9-based and other genetic manipulations: e.g.
  - Identification of mechanisms underlying the secretion of hormones from gut endocrine cells
  - Mapping protein-sensing mechanisms/circuits in the hypothalamus and defects in obesity
  - Epigenetics/imprinted genes and development pathways linked to growth and metabolism
  - Investigation of role and function of lipid droplet proteins and their defects in lipodystrophies
### APPENDICES A1. Translational Research at IMS-MRL

#### A1.3. Overview Matrix of IMS-MRL Scientists Involvement in Key Translational Research Activities

<table>
<thead>
<tr>
<th>Research Areas in Metabolic Health and Diseases</th>
<th>Translational Research Activities</th>
<th>Clinical Translational Research</th>
<th>Preclinical Translational Research</th>
<th>Fundamental Discovery Science</th>
</tr>
</thead>
<tbody>
<tr>
<td>- Genetics of human disorders of energy balance and insulin action (including rare diseases: severe obesity or thinness, severe insulin resistance, lipodystrophy)</td>
<td>FAROOQI group O’RAHILLY group SAVAGE group</td>
<td>COLL group FAROOQI group O’RAHILLY group SAVAGE group YEO group</td>
<td>COLL group FAROOQI group O’RAHILLY group SAVAGE group YEO group</td>
<td></td>
</tr>
<tr>
<td>- Thyroid disorders (including genetics of resistance to thyroid hormone, tumours of pituitary &amp; adrenals)</td>
<td>CHATTERJEE group GURNELL group SCHOENMAKERS group</td>
<td>SCHOENMAKERS group</td>
<td>CHATTERJEE group SCHOENMAKERS group</td>
<td></td>
</tr>
<tr>
<td>- Lipotoxicity and insulin resistance</td>
<td>O’RAHILLY group SAVAGE group</td>
<td>FAZAKERLEY group OZANNE group SAVAGE group VIDAL-PUIG group</td>
<td>FAZAKERLEY group SAVAGE group VIDAL-PUIG group</td>
<td></td>
</tr>
<tr>
<td>- Regulation of energy homeostasis</td>
<td>FAROOQI group O’RAHILLY group</td>
<td>COLL group FAROOQI group O’RAHILLY group VIDAL-PUIG group YEO group</td>
<td>COLL group FAROOQI group O’RAHILLY group VIDAL-PUIG group YEO group</td>
<td></td>
</tr>
<tr>
<td>- Cachexia and the anorexia of systemic disease</td>
<td>COLL group O’RAHILLY group</td>
<td>COLL group O’RAHILLY group</td>
<td>COLL group O’RAHILLY group SAVAGE group YEO group</td>
<td></td>
</tr>
<tr>
<td>- Brain pathways regulating appetite &amp; satiety and blood glucose</td>
<td>EVANS group FLETCHER group</td>
<td>BLOUET group EVANS group GRABENHORST group GRIBBLE - REIMANN gps MERKLKE group YEO group</td>
<td>BLOUET group EVANS group GRABENHORST group GRIBBLE - REIMANN gps MERKLKE group YEO group</td>
<td></td>
</tr>
<tr>
<td>- Regulation of the gut-brain axis</td>
<td>GRIFFBLE &amp; REIMANN groups</td>
<td>GRIFFBLE &amp; REIMANN groups</td>
<td>GRIFFBLE &amp; REIMANN groups</td>
<td></td>
</tr>
<tr>
<td>- Type 1 diabetes - hypoglycaemia - gene susceptibility - artificial pancreas (for type 1 &amp; type 2 diabetes)</td>
<td>DUNGER group EVANS group HOVORKA group MEEK group</td>
<td>DUNGER group EVANS group</td>
<td>DUNGER group EVANS group HOVORKA group</td>
<td></td>
</tr>
<tr>
<td>- Diabetes and metabolic dysregulation in pregnancy &amp; neonates</td>
<td>KOULMAN group MEEK group OZANNE group</td>
<td>CONSTANCIA group KOULMAN group MEEK group OZANNE group</td>
<td>CONSTANCIA group KOULMAN group OZANNE group</td>
<td></td>
</tr>
<tr>
<td>- Nutritional &amp; behavioural intervention strategies</td>
<td>FAROOQI group FLETCHER group MARTEAU group MEEK group O’RAHILLY group</td>
<td>CONSTANCIA group OZANNE group</td>
<td>CONSTANCIA group OZANNE group</td>
<td></td>
</tr>
</tbody>
</table>
APPENDICES A1. Translational Research at IMS-MRL

A1.4. Translational Research "Discovery Cascade" at IMS-MRL

Schematic of the conceptualisation of the “discovery cascade” of IMS-MRL Translation Research in the context of the Drug Discovery Research & Development cascade.

From BENCH

Disease Biology → Target ID → Target Validation Assays → Screening Cascade “Hit to Lead” → Lead Optimisation → Preclinical Candidates Development → Clinical Therapeutic Candidate Development

To BEDSIDE

And Back To BEDSIDE

IMS-MRL Expertise & Capabilities

Metabolic diseases mechanistic insights, including from genetics of extreme metabolic disorders in humans

In vitro and in vivo target validation models (e.g. genetically-engineered models)

IMS-MRL Expertise & Capabilities required from Pharma/CRO1

Efficacy in animal disease models and in human cell models
Pharmacokinetics
Selectivity; Toxicology
ADME2; CMC3; Scale-up
Regulatory

FIH/Phase 0
Phase 1
Phase 2a (Phase 2b)
Phase 3

1 CRO: Contract Research Organisation
2 ADME: Absorption, Distribution, Metabolism and Excretion
3 CMC: Chemistry, Manufacturing and Control

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APPENDIX 2: Overview of Translational Research Support at the University of Cambridge
- 2 pages -

- **Cambridge Academy for Therapeutic Sciences (CATS) -** www.ats.cam.ac.uk
  “Strategic research initiative to facilitate the development of new therapeutics” / “Home also to the Cambridge Alliance on Medicines Safety” / “Delivers also training and research programmes to support the education and training of the next generation of scientists and clinicians”
  
  **Key contact:** Dr Cathy Boucher (cathy.boucher@admin.cam.ac.uk)

  → “Gateway” first contact for preliminary discussion on ideas, even at a very early stage, and get some guidance, some info on training, or for any other information, such as when/who/how to contact other University bodies supporting translational research at different stages

  → Includes **Industry Experts in Residence** who visit University on a regular basis
    - Advice of applicability of specific ideas to drug discovery, diagnostics and devices
    - Advice on Translational Research grant applications
    - Advice on Regulatory aspects
    - Discuss progression of ongoing translational research projects
    - Works in conjunction with Cambridge Enterprise

  → **Monthly translation research seminar series**

  → **Translational research concept-development funding sources**

- **Cambridge Enterprise -** www.enterprise.cam.ac.uk
  “Supports University staff in achieving knowledge transfer” / “Help to use commercial avenues to develop ideas and expertise” / “Help with consultancy services - and with the protection, development and licensing of ideas, and with seed funding and new company creation”

  **Key contact:** Dr Amanda Wooding (Amanda.Wooding@enterprise.cam.ac.uk)

  → Includes **CUTS (CU Technical Services Ltd) subsidiary** which deals with consultancy contracts
    - Admin support for consultancies, including professional indemnity cover
    - Intellectual Property Management and advice on protection/disclosures
    - Help with writing strong patents and managing disclosures
    - Help with assessment of commercial potential and development of business plans
    - Management of licensing contracts
    - Connections to experts, mentors and investors (including Cambridge Enterprise as investor)

  → **Online confidential disclosure forms** can be filled and sent through the website or by email (disclosures@enterprise.cam.ac.uk) for any idea, invention, consultancy or other commercial opportunity. **Enquiry for an initial informal chat can also be made through the website**

  → **NB:** - The University as the employer of any inventor (or potential inventor) is the owner of his/her Intellectual Property and has the initial right to apply for a patent — therefore any potentially patentable technology has to be disclosed to Cambridge Enterprise.
    - A public disclosure is any non-confidential communication of a technology or idea; this could be a publication, a PhD thesis, an abstract, poster or talk, information posted online, or a publicly available abstract of a grant proposal, or a grant final report.
    - The evaluation of what is novel and patentable within the prior art of a field, the drafting and writing of patents, and their maintenance and prosecution, requires expertise and can be costly – always ask for help and advice!

  → **Commercialisation Surgeries** (weekly & monthly services)
    - **Technology Transfer Experts:** Advice on identifying commercial opportunities, matters relating to Intellectual Property (how to protect and licence IP) and provide information on starting your own company (sources of funding and when to form a spin-out)
    - **Consultancy Services Team:** Advice on matters relating to the benefits of sharing knowledge as a consultant, providing details on how Cambridge Enterprise can help facilitate your project.
(A2. cont.)

- **Office for Translational Research** - https://otr.medschl.cam.ac.uk/
  
  “To convert ideas from research emanating from the University and associated hospitals into the development of new products and approaches to treatment or prevention of human disease and illness”

  Key contact: Head of OTR: Dr Anita Marguerie de Rotrou (am2444@medschl.cam.ac.uk)

  → **Supports researchers from Cambridge University Health Partners (CUHP)** in:
  - Translating Research
  - Industrial Partnership development and coordination
  - Project Management

  → **Can be viewed as “internal consultants”** to help with:
  - Connecting researchers to internal and external resources and expertise and supports researchers in securing translational funding, whether institutional or industrial
  - Project planning and costing and devising protocols, document development, grant applications and submissions to Ethics Committees and regulatory bodies and reports for funders. Involved in Experimental Medicine Training Initiative.

- **The Milner Therapeutics Institute** - www.milner.cam.ac.uk

  “Aims to transform pioneering science into therapies / Based on bridging the gap between academia and industry / Aim at developing own drug discovery pipeline - focus on oncology”

  Key contact: Alison Schuldt (a.schuldt@milner.cam.ac.uk)

  → **Four Research Units:**
  - Centre for Pathway Analysis (including labs, target identification & validation)
  - **Start Codon accelerator programme** (including seed funding and mentoring)
  - AstraZeneca-Cancer Research UK Functional Genomics Centre
  - Cambridge Centre for Proteomics

  → **Housing start-up companies**, including with pharma partners

  → **Help with raising venture capital to invest in and support start-ups**

  → **Milner Therapeutics Consortium**: Research agreement between the University, the Sanger Institute and the Babraham and seven pharma companies which have set aside funds for collaborative projects in any therapeutic area (and including for post-doc funding) → more than 20 early stage projects across the University. (NB: Scientific Advisory Board members include Steve O’Rahilly, Director of IMS-MRL)

- **Some additional “entrepreneurial” support services for Post-Docs at Cambridge University**

  - “Entrepreneurial Postdocs of Cambridge” : www.epoc.group.cam.ac.uk
  - “Annual Postdoc Business Plan Competition” (Cambridge Enterprise: www.enterprise.cam.ac.uk/for-the-university/start-a-company-or-social-enterprise/the-chris-abell-business-plan-competition/)
  - “Postdoc Academy Cambridge” - Various “entrepreneurial” training events www.postdocacademy.cam.ac.uk/events

    www.p2i-network.eu : events and online courses run by “Postdocs to Innovators” network

    www.iteamsonline.org : i-teams organisation with cross-disciplinary courses and projects, including Medical i-teams in partnership with CATS www.iteamsonline.org/medical-i-teams/
APPENDICES A3. Templates for Translational Science Project Proposals

A3.1. Preliminary Project Proposal “Road-Map”
(for internal use) - 2 pages -
APPENDICES A3. Templates for Translational Science Project Proposals

A3.2. Non-Confidential Brief Project Proposal Outline
(to engage with translational research supporting bodies or with potential external partners)

This template could be used as a guide-line for the preparation of a brief “official” (1-2 page document with appropriate logos) Non-Confidential Translational Research Project Proposal Outline highlighting key points that should maximise interaction with the different Translational Research supporting bodies or schemes, and stimulate the engagement of potential biotech/pharma collaborators or other external partners (e.g. from complementary “discovery” fields such as chemistry, pharmacokinetics, toxicology, regulatory, established in vitro / ex vivo assay platforms or preclinical Contract Research Organisations).

- Descriptive title
- Project overview (short and clear “executive” summary)
- Vision and key objectives
- Brief background on the main focus area (with some key publications)
- Proof-of-Concept with key supportive references
- Current stage of project development / key points of validation obtained to date
- Preliminary Freedom-to-Operate analysis and Intellectual Property assessment
- Operational feasibility / Evidence of relevant and cutting-edge skills, experience and facilities
- Experimental design overview and key measurable end-points
- Key outcomes and potential significant impact
- Novelty and competitive edge / Competitor status
- Project plan & strategy with critical milestones and decision points / Resources & time-lines
- Collaboration or partnership opportunity
  What type of collaboration or partnership and level of involvement expected? Research funding? Research and Development partnering? Pharmaceutical collaboration / acceleration / cross-expertise?
- SWOT analysis of project content and approach
  Strengths: e.g. skills, experience and access to facilities to deliver the project, innovative approach
  Weaknesses: e.g. any gap within the step-by-step work plan, limited access to some resources
  Opportunities: e.g. potential significant outcome to exploit
  Threats: e.g. uncertainties such as ethical issues, sufficient funding, unknown competition