



ITI Life Sciences

**Call for Expressions of  
Interest**

**Intra-Ocular Delivery  
of Biologics**

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# Call summary

**As a division of ITI Scotland, ITI Life Sciences (ITI LS) is a company focused on driving innovation in the Life Sciences sector. With a total fund of £150 million we seek to commission and deliver outstanding R&D programmes. With this call, ITI LS is aiming to identify innovative R&D opportunities with the potential of generating IP that will enable intra-ocular delivery of biologic molecules and drive future markets in ophthalmic therapeutics and diagnostics.**

ITI's aim is to create new commercialisable technologies and stimulate business growth in Scotland, through establishing and funding international collaborative research programmes. We identify future global market opportunities and create commercially driven research programmes to address future industry needs. To date, we have committed over £50 million in five research programmes in the fields of stem cell technologies, text mining, transgenic screening and safety models, cardiac biomarkers, and lifetime fluorescence assays. Most recently we have instigated a drug discovery programme around the Ubiquitin Proteasome System.

Central to the ITI mission is that the value from these research commitments is achieved through the creation of foreground Intellectual Assets (IA) held by ITI and that these will subsequently be taken to the markets through licensing relationships with existing or new companies. Therefore, any ITI funded research programme must have the potential to generate valuable foreground IA.

This Call for Expressions of Interest (CEI) arises from an extensive series of Foresighting activities that ITI undertook, in order to identify areas of high unmet need requiring innovative, disruptive technology.

Recently ITI has released a series of detailed market and technology Foresighting reports covering the areas of Molecular Imaging, Drug Delivery Technologies, Nanomedicine and Therapeutic and Diagnostic Antibodies. These analyses, while detailing specific opportunities in each of these areas, have also led to ITI's current interest in the cross-over opportunities that are at the heart of this Call.

Thus, we believe there are many unmet medical needs requiring early diagnosis and treatment of ophthalmic diseases and that potential solutions will be built upon new and novel technologies that enable the intra-ocular delivery of biologic molecules. As a result of these conclusions, ITI Life Sciences is keen to identify R&D opportunities, with the

potential of generating IP, to form the basis of a research programme related to the delivery of biological agents to the interior compartments of the eye. As a general guide, ITI Life Sciences typically commissions programmes in the range of £3-10 million (\$6-20 million) with an average lifetime of three years.

## **Why respond to this call for expressions of interest from ITI LS?**

ITI provides more than just finance. We have a proven track record of identifying exciting new market opportunities and of working with entrepreneurial companies and academic groups to create real innovation with a commercial focus. In supporting R&D programmes ITI LS will:

- Cover the full costs of delivering our programme
- Appoint experienced programme managers to coordinate delivery of programme goals and oversee the research
- Undertake real time commercial and competitive landscape analysis as the programme progresses, to retain a competitive edge
- Provide access to outstanding scientific, marketing and commercial expertise via global networks

Responses describing your relevant expertise in the area of intra-ocular delivery of therapeutic and diagnostic biologic agents, and where you see potential for innovation, are invited from companies, agencies, institutes, academia and individuals. In return you could have the opportunity to participate in an international cutting-edge collaborative R&D programme. Moreover, once intellectual assets are generated through the programme we will be looking for partners able to maximise the commercialisation of our assets in the market place.

Call responses should be emailed to [IOD@itilifesciences.com](mailto:IOD@itilifesciences.com) no later than the 2<sup>nd</sup> June 2008.

# Funding rationale

## **Market & Technology analysis reveals an opportunity for innovation and commercialisation in a field of rapidly growing unmet medical need.**

Through Foresighting activities, ITI has built up an extensive knowledge and insight into the ocular therapeutic and diagnostic markets. ITI has been drawn to a market segment that has significant unmet diagnostic and therapeutic needs. The area could benefit dramatically from the creation and development of broad enabling technologies for the delivery of biologic-based drug or diagnostic agents to the eye. This market segment represents the growing population of patients suffering from a wide spectrum of ocular diseases leading to vision impairment and, in some cases, blindness. The prevalence of these conditions is growing rapidly as a result of both a generally ageing population and dramatic increases in the occurrence of obesity and diabetes.

Despite these worrying medical trends, current diagnostic detection and therapeutic interventions remain largely palliative, at best delaying disease progression but having little impact on eventual loss of vision and blindness. We believe that the paucity of innovative product launches in this segment is in part due to the lack of effective, non-invasive technologies that enable intra-ocular delivery of active agents.

## **There is a funding need for the development of next generation platforms for intra-ocular delivery**

ITI believes there is a window of opportunity for the development of technologies that can enable safe and effective delivery of new products in the ophthalmic market, both in diagnostics and therapeutics. There are still few non-invasive proprietary delivery systems that effectively overcome the physical and physiological barriers to reaching the affected tissues in the eye interior (e.g. lens, vitreous, and retina). In addition, biopharmaceuticals approved for systemic delivery in other disease indications (e.g. targeting TNF or VEGF) could provide additional opportunities for the treatment of eye conditions if appropriate delivery systems were available.

The additive effects of age and diabetes will have a dramatic impact on visual health in the next three decades and result in an increasing need for both effective diagnostic and therapeutic modalities. It is estimated that, currently, few patients receive appropriate therapies due to the invasive nature of delivery procedures, high cost, adverse reactions and poor response rates. It is anticipated that more effective treatments, administered in GP clinics and health centres, will dramatically increase the proportion of existing patients who receive treatment. By treating conditions earlier, with more effective therapies, healthcare providers will aim to delay or prevent having to undertake costly treatment of advanced symptoms in later life.

## **Why Biologics-orientated delivery platforms?**

The programme specifically targets the ocular delivery of biologics, whether as therapeutics or as diagnostic agents. Many biologics have now gained therapeutic approval for a wide range of non-ocular, chronic indications in therapeutic areas including inflammation, oncology, metabolic and degenerative diseases. Some of these have subsequently been approved for ophthalmic indications such as age-related macular degeneration and diabetic retinopathy. These include approved protein drugs such as Lucentis®, oligonucleotide aptamer drugs such as Macugen®, and antisense RNA molecules such as Vitravene®. Our research has also identified a number of other biologics currently progressing through pre-clinical/clinical pipelines, including protein, gene- and cell-based therapies and drugs based on siRNA.

Over recent years diagnostics in general have benefited tremendously from a focus on 'molecular diagnostics' and the ability to identify and measure very specific analytes or biomarkers. We believe that there will be similar benefits expected for ocular diseases in the near future and that many new analytes or biomarkers will be identified and validated, becoming important predictive or early indicators of disease. ITI believes that it is likely that the best tools for analysing these markers will be based on biologics; for instance antibodies have become very powerful and widely applicable diagnostic agents. Further evolution of diagnostic probes has included antibody fragments as well as a diverse mix of reagents, including nucleic acid or peptide based aptamers.

ITI believes that non-invasive ocular delivery platforms designed especially for macromolecule-based drugs are currently at a relatively early stage of maturity despite the diversity of approaches that have already been considered (e.g. use of biopolymer and nano-particulate entities). Hence, there are considerable opportunities for innovation in this area.

# Where we see the opportunities

Delivery of biologics into the eye currently relies on intra-ocular injections, or systemic administration. These approaches have significant drawbacks to achieving both effective and safe delivery to the eye. We believe a delivery method that enables non- or minimally-invasive delivery of biologicals to the interior of the eye would have great benefit to patients and healthcare providers. The key criteria for success for such a delivery method are: patient acceptability, ease of administration, effective dose, side-effects, cost-effectiveness, treatment interval, speed of delivery, and reproducibility.

We are keen to identify experts and innovators, like you, that can come together with ITI to scope how the challenges of delivering biologics to the eye can be overcome and what experience and expertise you can bring to a programme in this area. We invite responses from innovators in the following areas:

- Ophthalmology
- Nanotechnology / nanoparticles
- Optical instrumentation/ fluidics
- Protein / peptide delivery
- Drug Delivery
- Microspheres
- Electroporation / iontophoresis
- Medical devices
- Biopolymers

While this programme will not be intending to support development of specific new unproven therapeutic agents, we are, however, interested in using specific test models to validate our approaches. Thus, we would like to hear from researchers willing to provide access to particular, clinically validated, biologics that we can test in delivery to the eye.

## We are NOT currently looking to fund:

- A programme that will only be capable of delivering small molecules.
- Therapeutic agents (NCEs) still at the research stage, whether biologic or small molecule.
- Projects seeking to develop systemic delivery of ocular diagnostics or therapeutics.
- Our primary driver is the generation of IA and we are therefore unable to fund the development of what might be a very exciting technology platform but which has limited scope for generating new IA. Additionally, we do not provide funding for 'blue sky' research.

# How to respond

To guide and standardise responses, a template is provided at [www.itilifesciences.co.uk/IOD](http://www.itilifesciences.co.uk/IOD). Within the template there are two main sections, both of which must be completed with as much relevant detail as can be provided.

## Section A

An outline of what you believe you could bring to a programme in this area. This should detail your relevant experience and innovation track record (utilising no more than 1500 words).

## Section B

A non-confidential outline of where you see the need for innovation in this area. We are not seeking specific approaches or technical solutions but more a short discussion of where you see the challenges and trends and the type of skills required that should be brought together to create a new programme (utilising no more than 1500 words).

Responses will be evaluated according to:

- Track record in delivering innovative new technology in area of expertise
- Demonstration of thought leadership in that could contribute to a programme in this area
- Potential for enabling the development of convergent, disruptive technology

Call responses should be emailed to [IOD@itilifesciences.com](mailto:IOD@itilifesciences.com) NO LATER than the 2<sup>nd</sup> June 2008

Responders may be invited to discuss or provide further clarification on their submission in person or via teleconference.

ITI reserves the right to explore other opportunities within the Intra-Ocular Delivery field that may arise as a result of activities that are distinct from this particular Call for Expressions of Interest.

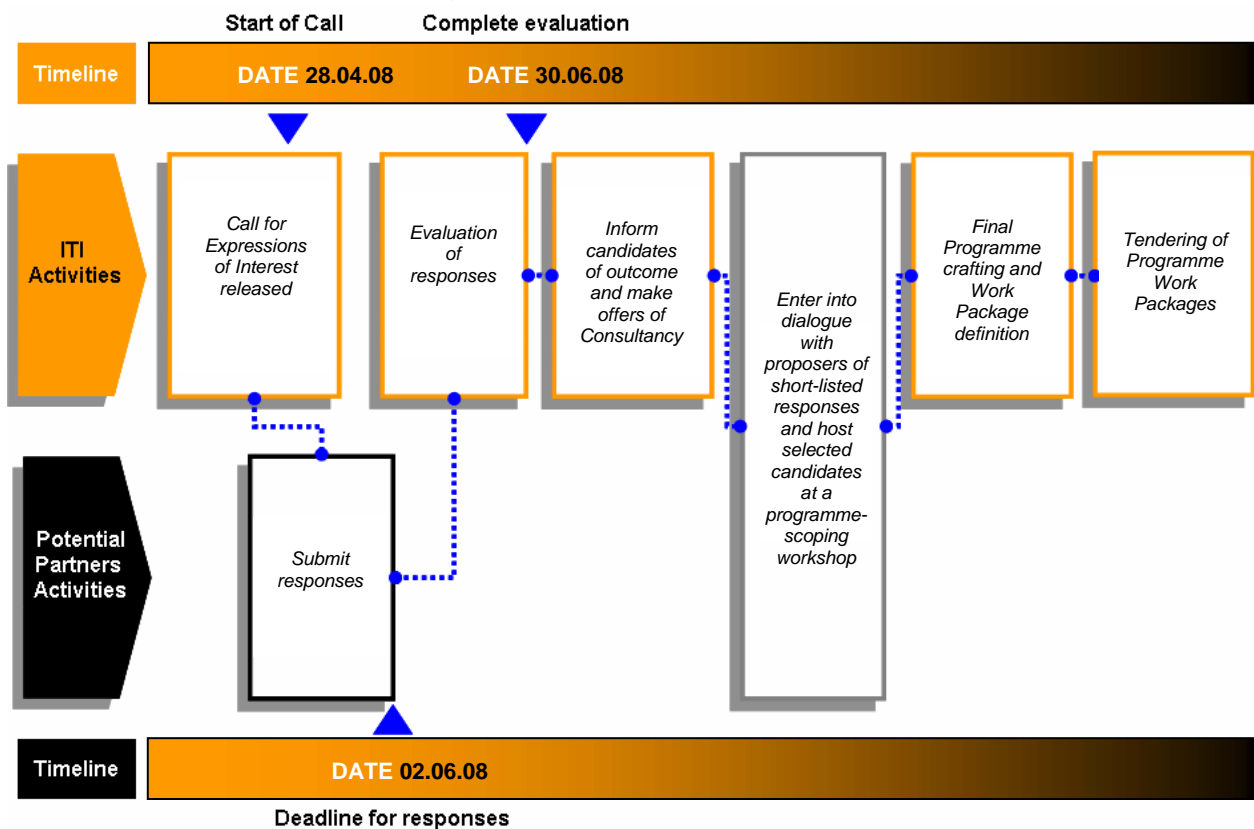
Please direct all questions regarding this call to [IOD@itilifesciences.com](mailto:IOD@itilifesciences.com)

# Process and schedule

Responses will be evaluated by ITI LS and the initially selected responders will be invited to participate in a workshop(s) convened by ITI LS. The purpose of this workshop will be to synergise the creativity of the participants to identify a focus area for innovation. Attendees will be participating as retained ITI LS Consultants. Hence, ITI LS will conclude a contractual consultancy arrangement with the selected attendees in advance. This agreement will set out their consultancy fee, travel and subsistence expenses, CDA and assignation of any IA generated at the workshop to ITI LS.

It is planned that that the focus area selected will then be iteratively developed into a programme via the Consultants, other selected responders and newly targeted expertise groups, as appropriate. A collaborator network will then be identified and formed to execute the programme.

Subsequent steps prior to programme initiation are also outlined below.





# ITI Life Sciences

## Overview

# ITI Life Sciences Overview

Established in 2003, ITI Life Sciences is one of the three Intermediary Technology Institutes that make up ITI Scotland. ITI Life Sciences is focused on driving innovation in the Life Sciences sector, through successful and creative investment of up to £150 million. We identify future global market opportunities and create commercially driven research programmes.

We actively manage programmes with a long term view and look to transcend traditional scientific boundaries with our Research Providers and Commercial Partners to generate market-focused intellectual assets for exploitation by existing and new companies. Our people are highly skilled and mainly drawn from the commercial sector with specialist life sciences expertise. Our investments are based on numerous selection criteria, including the potential for generating new IA, and are intended to generate financial returns and have an economic impact within Scotland. We do not provide grants or fund basic or blue sky research.

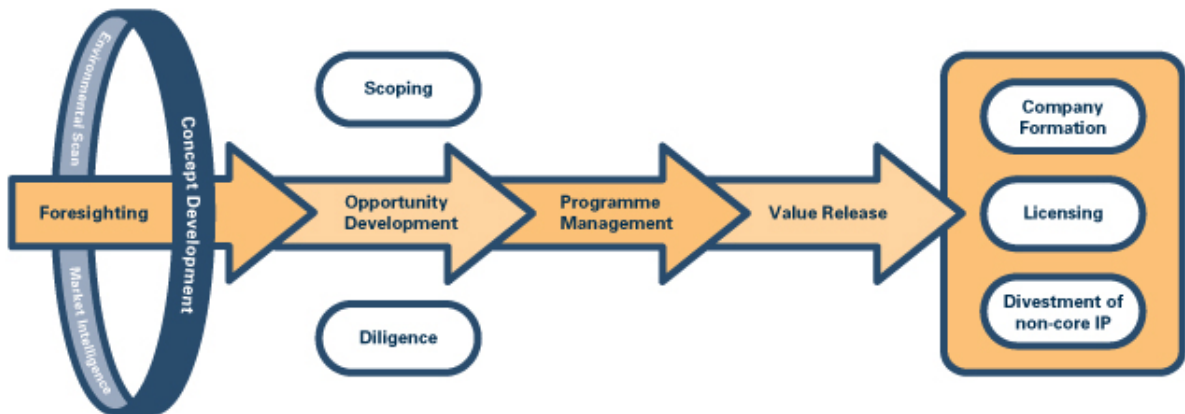
To date, we have committed over £50 million in 5 research programmes in the fields of: stem cell technologies; text mining; transgenic screening and safety models; cardiac biomarkers; and lifetime fluorescence assays. We have also produced a range of market Foresighting reports and have a pipeline of future opportunities. In addition to the ongoing benefits of our research programmes, we have an active, international membership of business, research, academic and public sector organisations, who enjoy exclusive access to our market research reports and networking opportunities.

Further information is available on our website ([www.itilifesciences.com](http://www.itilifesciences.com)), along with information on our existing R&D Programmes.

The ITI operating model is illustrated below. Beginning with a rigorous process of market foresighting and technology assessment, ITI identifies and reviews programme opportunities.

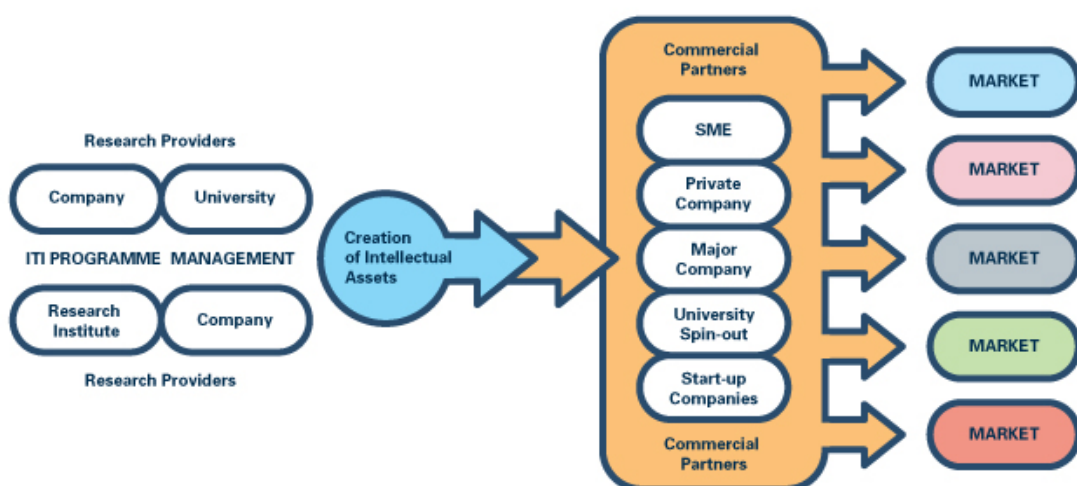
The most attractive opportunities are developed into programmes, which are managed by ITI, with the aim of generating novel foreground Intellectual Assets (IA) which are owned by ITI LS.

During the final step, ITI manages the commercialisation activity through the release of IA, normally through the formation of a company or via licensing.



ITI works with interested parties to identify the most suitable candidates for a programme. This includes carrying out due diligence, negotiating contracts, and securing the rights to use background IA, where relevant.

Collaboration is at the heart of our operating model. There are several ways for companies or organisations to become involved in a potential programme:



Research Providers: provide technical expertise and are contracted at commercial rates to develop specific IA. They can be companies, universities or research institutes.

Commercial Partners: invest some combination of commercial and technical expertise, finance, resources, facilities and potentially also background IA (for which market value will be given). Partners can also be potential licensees of new IA created and/or users of resulting products.

Other Licensees: much of the IA created will have the potential to be exploited in other fields of use. We will actively seek potential licensees for these technologies and welcome approaches from organisations interested in licensing opportunities.

# Who ITI works with:

