Written evidence from Kidney Research UK (ENB0020)

Kidney Research UK

Kidney Research UK is the leading kidney research charity in the UK. We want to prevent kidney disease by finding ways to see it coming and stop it happening; protect people from reaching kidney failure by learning how to spot disease early, halting disease progression, and repairing kidney damage; and transform treatments by making dialysis more tolerable and making transplants last longer until better alternatives are available.

There are an estimated 7.2m people living with kidney disease in the UK and the numbers are growing. Kidney disease costs the UK economy £7bn annually, with a direct cost to the NHS of £6.4bn. These costs could rise as high as £13.9bn and £10.9bn respectively if we don't do more now to prevent people reaching kidney failure. Kidney disease has a significant human, economic and environmental cost worldwide.

We have submitted evidence to this inquiry as engineering biology offers significant opportunities for the advancement of kidney interventions and enhanced patient outcomes.

What are the UK's key strengths in the area of engineering biology?

- **Are there any notable research institutes or groups or key projects? Are there innovative companies, start-ups, or spin-outs that you think are of particular promise or significance using engineering biology in the UK today?**

Purespring Therapeutics is the world's first kidney gene therapy company, with £45m series A funding and a plan for first in human trials by the end of 2025.

- **What is the current economic impact of engineering biology on the UK and what might its potential economic impact be?**

There has been inward investment from pharmaceutical companies and several venture capitalists (VCs) to Purespring, currently at late stage of discussing terms. The total investment is approximately 500m Euros.

2. What are the key applications for engineering biology?

- **Can you give examples of particularly exciting or interesting applications? In particular, applications which could be taken forward or are being worked on in the UK?**

Gene therapy, as above. Several groups (e.g. UCL, Bristol, Sheffield) are working on nano body engineering at the academic stage.

- **On what timescales might the different applications for engineering biology be realised? Which applications are emerging now, and what is on the horizon in the next 5–10 years or further ahead?**

For gene therapy, there are two programmes to first in man in 2025/6.

- **Are there areas of application for engineering biology where the hype exceeds the reality, or where significant barriers remain?**

Stem cell and kidney bioengineering faces significant barriers due to the complexity of kidney architecture and differentiation.

- **How does the UK compare to other countries, such as Germany, the US, or China, in terms of investment and policy activity, as well as areas of specialism?**

The UK currently sits below the US and Europe for VC funding for biotech.

3. How can Government policy support the development of engineering biology?

- **The Government has committed to spend £2 billion over the next 10 years on engineering biology. Is this scale of subsidy sufficient to be competitive? Where should this funding be focused to best support engineering biology in the UK? Is it more important to support facilities, skills, or flagship research projects? Which specific skills or facilities are most needed?**

A major gap is still the transition from academic results to commercialisation. Universities lack adequate personnel (with the requisite experience) to guide their successful academics. Funds to move to seed and Series A funding are inadequate.

Drug development (e.g. regulatory pathway, analytics, manufacture) is a specific skillset that is not broadly available to small companies.

It would be worth considering whether universities themselves can be upskilled to become the 'drug developer', instead of pharmaceutical companies, without the need for significant profits from the eventual product.

4. How can the UK maximise the economic potential of developments in engineering biology?

- **How should the Government best support engineering biology startups to scale-up in the UK? Are there specific facilities that it would be helpful to invest in? Are the financial support mechanisms for start-ups and scale-ups appropriate and sufficient, or could they be reformed?**

Financial support and availability of experts from industry (e.g. chemists, IP, manufacturing etc) needs boosting.

- **How well are Innovate UK, British Business Bank and British Infrastructure Bank supporting the commercialisation of engineering biology in the UK?**

Innovate UK is too limited in scope and focus. Projects are highly constrained and do not allow sufficient room for variation.

- **Does the UK need large companies in the field to help form the ecosystem in which spinouts and start-ups can thrive? If so, does it have the right ingredients for a healthy engineering biology ecosystem? Are major industrial players investing in engineering biology?**

A better interface between pharmaceutical companies and spinouts needs to be created. Currently the system is ad hoc, depending largely on personal contacts and serendipity.

- **What can the Government do to encourage investors to invest in engineering biology and is there a need for investors with more scientific expertise?**

Large VCs usually have good scientific expertise. Ideally, they should be more willing to take risks, and also invest in projects without the multiples on returns that they currently expect. This would need back up for investments that fail.

- **How does the UK's approach to engineering biology, commercialisation and translation compare to other nations, such as Germany, China and the US? Are there specific areas the UK should look to focus on in order to gain or maintain a competitive advantage?**

We have world leading academia in kidney medicine, but relatively poor commercialisation outputs.

Projects currently funded by Kidney Research UK related to organoids, gene therapy and stem cells:

Summary	**Institution**
Engineering primary kidney organoids from the human amniotic fluid to model renal development and disease	Royal Free Hospital and UCL Medical School
Using human organoids to understand cardiovascular complications in children with chronic kidney disease	University College London (UCL)
Developing splice-switching oligonucleotides as therapeutics for CEP290-related renal ciliopathies in organoid models	University of Leeds
Integrin beta-1 as trigger for the lectin complement pathway in acute kidney injury	King's College London

Role of PLCg1 in kidney disease and metabolism	Brunel University London
Human Renal Tubular Organoids for Polycystic Kidney Disease Modelling and Drug Screening	University of Cambridge
Investigating the role of TMEM260 in renal development and disease.	University of Oxford
Repairing basement membranes in Alport syndrome	University of Manchester
Pre-clinical testing of ROCK2 inhibition as a new therapeutic treatment for cystic kidney diseases	University of Leeds
Repair of human kidneys using neonatal kidney stem-/progenitor cells	University of Cambridge
Organoid models for assessment of novel therapeutics against BK polyomavirus	University of Cambridge
Targeting the epigenome as a new therapy for diabetic kidney disease	Bristol Royal Hospital for Children

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