


The Realisation of Research





The trials and tribulations of running a TTO

What's the end game?

Cengiz A. Tarhan

Managing Director

UCL Business PLC

November 2017

UCL – London's Global University



- ❑ Established over 180 years ago
- ❑ Today – 980 professors and 6000 academic/researchers
- ❑ £1.4 billion annual turnover
- ❑ Ranked **7th** in the QS World University rankings for 2017 and **16th** in the Academic Ranking of World Universities - 2017 (**3rd** in Europe)
- ❑ Exceptional research strengths

UCL – Top in the UK for research power

UCL 2014 Research Excellence Framework (REF)

- ❑ UCL top in the UK overall (GPA x FTE)
- ❑ UCL top in the UK overall (%4* + %3*) research x FTE
- ❑ 1st - Most impactful research (GPA x FTE weighting)



Creation of UCLB – back in 1990's



And



Realisation – it's not so easy

- ☐ Needs good ideas – IP protection
- ☐ People who understand the science
- ☐ Money
- ☐ Partners – investors/collaborators
- ☐ Understanding development/regulatory pathways
- ☐ Time
- ☐ Patience – a lot

And plenty of luck!

We commenced in earnest

Incorporated as FreeMedic PLC in 1993

- ❑ 1995 - first spin out IPO (PolyMASC Pharmaceuticals PLC, latterly acquired by Valentis/Urigen Inc).
- ❑ 2003 – enlarged UCL BioMedica
- ❑ 2006 – enlarged UCL Business

Experimenting, learning and evolving for nearly 25 years

UCL Business – now

- ❑ Wholly-owned by UCL
- ❑ Focus on IP commercialisation
- ❑ Working closely with partner hospitals
- ❑ Financially self-sufficient
- ❑ Independent but closely aligned with UCL

A Technology Commercialisation Business – but why?

UCL Business is a business - why?

- ❑ TT process not well understood
- ❑ Leadership changes bring frequent reviews
- ❑ Universities like to experiment
- ❑ Its about people and teamwork
- ❑ Needs investment.
- ❑ Inherent conflicts between university, inventors, entrepreneurs/management, funding bodies, governments and investors/businesses
- ❑ It's a long term game - no guarantees

A company structure works for us

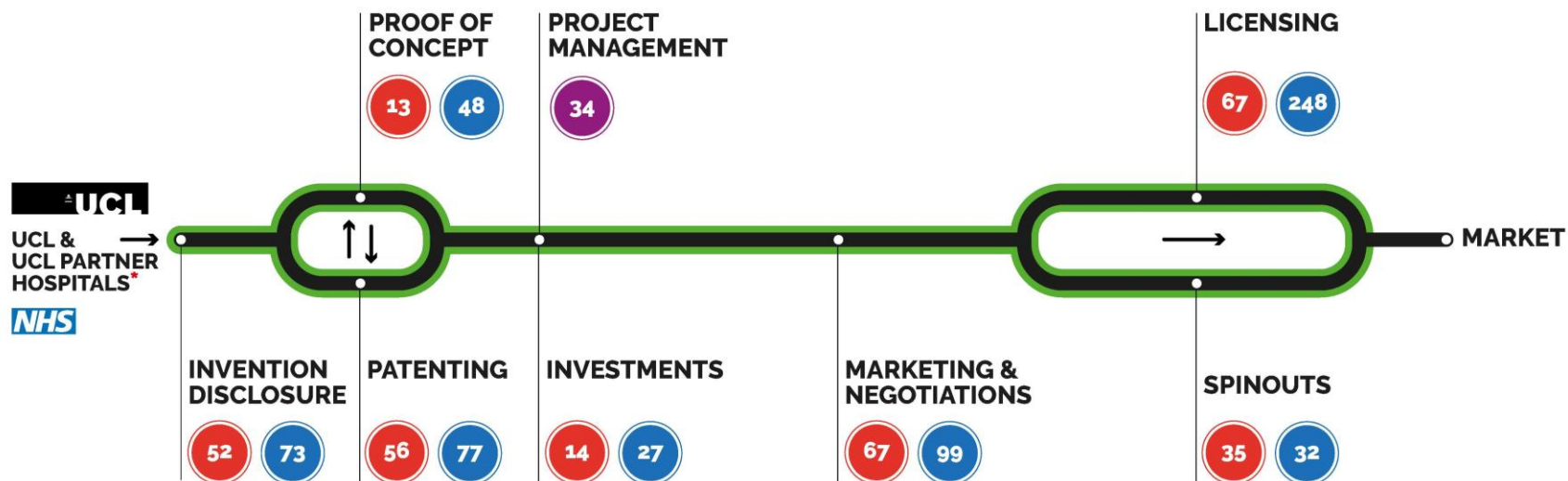
UCLB – the business now

- ❑ We identify, build and sell assets
- ❑ We invest – £50M UCL Technology Fund
- ❑ Focus on ‘demand pull rather supply push’
- ❑ Secure Translational grants and external funding
- ❑ Develop therapeutics for licensing – £40M Apollo Fund
- ❑ Create Spin Outs and support them
- ❑ Value and manage our portfolio of assets
- ❑ Deliver benefits to our shareholder

Like any other business

UCLB – pipeline and journey

OUR ROUTE TO MARKET



KEY

UCLB activity

Total number of active projects per phase

- Engineering, Physical Sciences, Arts & the Built Environment
- Biomedical Sciences
- Project Management

* Great Ormond Street Hospital for Children NHS Foundation Trust

Moorfields Eye Hospital NHS Foundation Trust

Royal Free London NHS Foundation Trust

University College London Hospitals NHS Foundation Trust

UCLB – previous licence

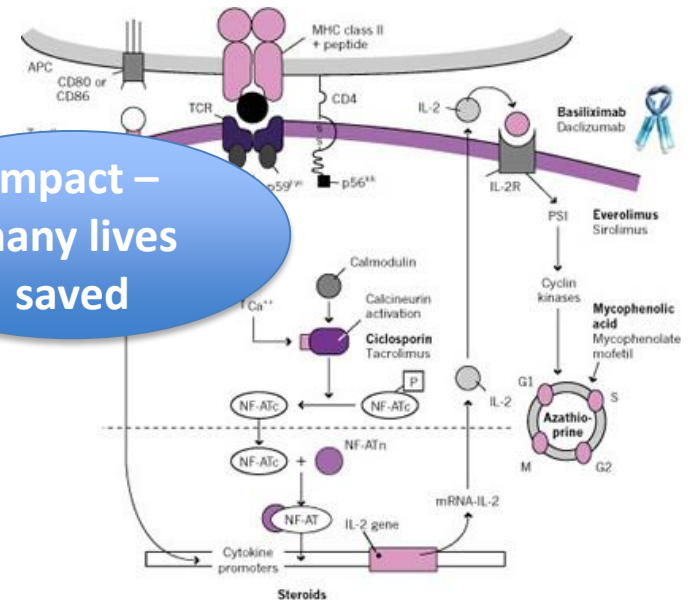


- ❑ Collaboration with Sandoz now Novartis.
- ❑ Product developed through Clinical trials Phase I-III/IV and launched as Simulect for use in renal transplantation
- ❑ Simulect works by blocking the receptor for IL-2, a protein that stimulates proliferation of T-lymphocytes, which play a key role in organ transplant rejection.

£30m in royalties
2000-2011



Impact –
many lives
saved



UCLB - previous UCL spin outs

- ❑ PolyMASC – first university spin out on UK AIM (1995)
- ❑ Ark Therapeutics – floated on LSE March 2004
- ❑ Arrow Therapeutics – sold to Astra Zeneca 2007 \$150M
- ❑ Map of Medicine – sold to Informa 2006
- ❑ Stanmore Implants Worldwide – sold to Abingworth 2008
- ❑ Spirogen – sold to Astra Zeneca 2013 \$200M +
- ❑ Pentraxin Therapeutics – in phase II collaboration with GSK
- ❑ Domainex – Med Chem service with £7M turnover/40 staff
- ❑ Biovex – Sold to Amgen for £1Bn

Delivering significant economic impact

UCLB – How the model works now to create more

- ❑ Therapeutic area - liver failure
- ❑ UCLB invested ≈£60K PoC funds to validate and secure IP position then committed £500K
- ❑ Secured £750K from a MRC Exper. Med Grant
- ❑ Licensed Ornithine Phenylacetate (OCR-002) to Ocera Therapeutics
- ❑ Phase 2a for oral application in progress
- ❑ Phase 2b intravenous completed successfully - FDA discussions continuing.



Work in progress

UCLB – Progress with Licences in portfolio

“FDA Grants Breakthrough Therapy Designation for BioMarin's Valoctocogene Roxaparvovec (formerly BMN 270), an Investigational Gene Therapy for Hemophilia A - Global Phase 3 Program to Begin Before Year End (26 Oct '17)

“New licensing agreement for UCL technology to combat antimicrobial resistance”
(28 September '17)

“TC BioPharm announces CAR-T licensing deal with UCL Business” (6 Sept '17)

“Ocera announces top-line results for phase 2b study in hepatic encephalopathy”
(6 February '17)

300 + active licences across all areas

UCLB – Recent ‘spin outs’ in portfolio

“Syncona LLP and UCLB Announce the formation of Syncona, a Cancer Immunotherapy Company”

“UCLB announces the launch of Freeline Therapeutics”

“Syncona and UCLB launch Freeline Therapeutics, a company focused on developing therapies for bleeding disorders”

“UCLB launches Intract Pharma Ltd”

“UCLB and F-Prime launch Orchard Therapeutics, a company focused on developing therapies for childhood diseases”

(Announced on 3 May 2016)

“UCLB Achilles Therapeutics launched with funds of £13.2m to develop immunotherapies for cancer”

(Announced on 5 October 2016)

Initial FUNDING

**FUNDING
SECURED TO DATE
£310 M +**

And more on the way !

UCLB – Creating businesses from UCL's Research



- ❑ Developing ex-vivo lentiviral gene therapy to restore normal gene function in patients with ADA-SCID
- ❑ Simply stated
 - ❑ When a child inherits a severe combined immunodeficiency (SCID) of the immune system.
 - ❑ Patients own cells are collected, genetically corrected and transplanted back
 - ❑ ADA SCID is characterized by a deficiency in the enzyme adenosine deaminase (ADA) and accounts for 10–15% of all cases of SCID.

UCLB – Creating businesses from UCL's Research

Orchard
therapy

- ❑ Develop gene function
- ❑ Simply
- ❑ When immune
- ❑ Patients and
- ❑ ADA enzyme 10-

GENE THERAPY SUCCESSES AND FAILURES

SCID is often called "bubble boy disease". SCID became widely known during the 1970's and 80's, when the world learned of David Vetter, a boy with X-linked SCID, who lived for 12 years in a plastic, germ-free bubble. He died after a bone marrow transplant.



A recessive disorder of a mutation in the adenosine deaminase (ADA) gene causes SCID. Gene therapy successfully replaced this gene in several ADA patients

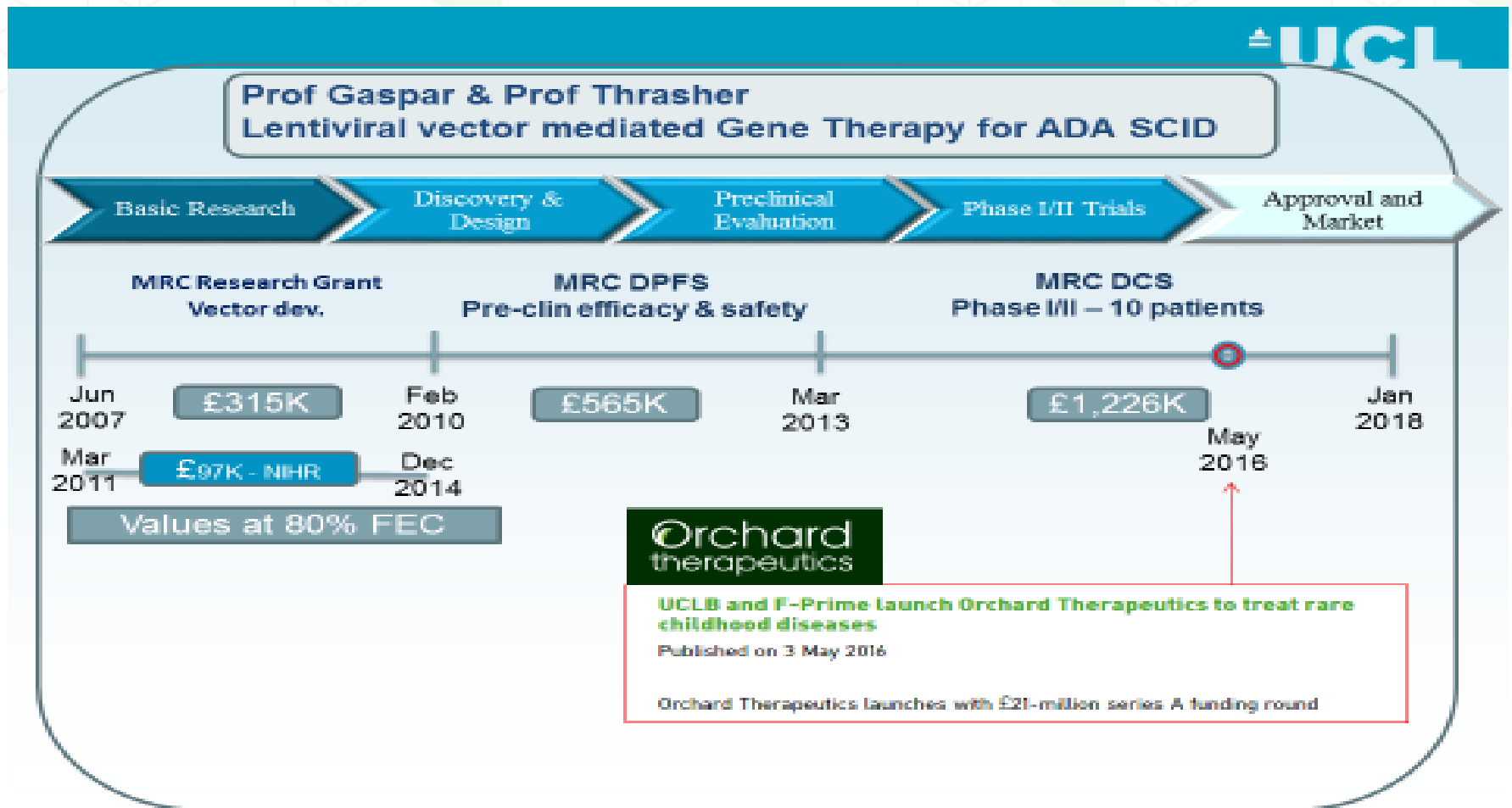
re normal

em.

corrected

the
counts for

UCLB – Creation of Orchard Therapeutics



UCLB – Creating businesses from UCL's Research



- ❑ Summary of clinical data:
- ❑ >30 patients treated at UCL and UCLA as of March 2016
- ❑ 100% survival with a follow-up of 2–49 months
- ❑ Evidence of immune reconstitution
- ❑ Commercial progress
- ❑ Secured £21M investment + second round
- ❑ August 2017 OTL-1 received Promising Innovative Medicine designation

UCLB – Funds for investment

UCL TECHNOLOGY FUND

“UCLB launches £50 million technology investment fund”

(Announced on 22 January 2016)



“Consortium of world-leading UK universities and global pharmaceutical companies launch £40 million fund to drive therapeutic innovation”

(Announced on 25 January 2016)

UCL Technology Fund

- ❑ Why create the UCLTF – because good projects need funding.
- ❑ Main differentiators
 - ❑ Independent Fund manager but UCLB is integral
 - ❑ UCLB is incentivised - receives fee and 'carry'
 - ❑ Proof of concept element
 - ❑ Invests in licence projects so not only spin outs
 - ❑ Term 10 -12 years

Progressing very well.

Apollo collaboration

- ❑ Three UK University TTO's and three Pharma companies
- ❑ Main components
 - ❑ £40M - all parties invest and all share returns
 - ❑ Independent drug discovery team appointed
 - ❑ No management fees
 - ❑ Licensing only
 - ❑ Expect to bolster partner pharma pipeline but no pre negotiated terms
 - ❑ IP remains with university TTO. Licence and terms negotiated by TTO at IND stage

UCLB – Is there an ‘end game’?

So why are we doing all this?

Provost's View: How we plan to secure our place in the world top 10



Context

UCL's world top 10 status enables:

- attracting the best minds
- increased government funding
- exciting partners from business and industry.

We want to maintain this status and take the next steps to make sure all our disciplines can be world-leading.

Currently, we are world-leading in around half our disciplines.

We need to innovate and take measured risks.

Listening and learning

Our planning processes enable us to anticipate the impact of further UCL growth and ensure that it doesn't harm teaching and learning nor the student experience.

Through constructive discussions with Council, we have agreed that it is appropriate to take more time to reach our financial targets. We are also aware that our professional services need to support the academic mission.

Later this year, the Transforming Our Professional Services programme will provide a number of quick wins and a clear plan for achieving transformation.

Our plans

The ideas that we plan to take forward do the following:

- demonstrate genuine, grassroots academic enthusiasm
- come from areas that have the potential to become world-leading
- are deeply cross-disciplinary.

The chosen ideas have different levels of academic vision and support at this stage of development.

Established ideas:

School of Economics and Public Policy
UCL East phase one

Emerging ideas:

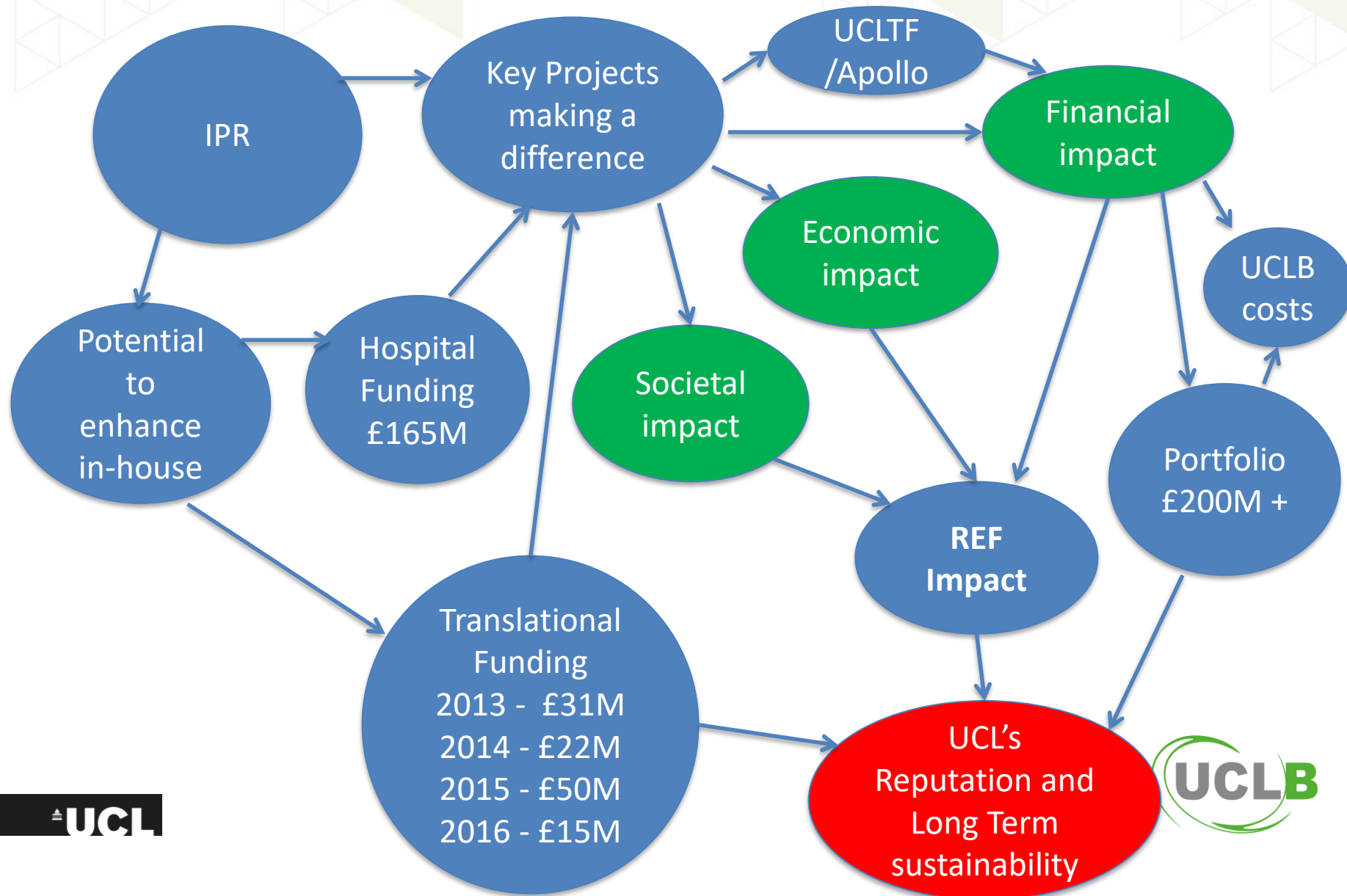
Institute for Mathematical
and Statistical Science

Early stage ideas:

School of Future Media and Communications/
Creative and Applied Humanities
Harwell for developments in physical and
space science

If there is something else that you would like
to know more about, please contact us:
provostfeedback@ucl.ac.uk

Our 'End Game'



Summary

- ❑ Your end game depends on Your University
- ❑ One size does not fit all
- ❑ Agree clear objectives
- ❑ Resource well – staff and funding
- ❑ Test frequently to align interests - not always easy.
- ❑ Demonstrate success with examples not just numbers
- ❑ Manage expectations – there will be failures
- ❑ Persevere – it's a long haul !!
- ❑ The end will be rewarding

The **Realisation** of Research

Thank you

Cengiz A. Tarhan

Managing Director

c.tarhan@uclb.com

UCL Business PLC

www.uclb.com



The **Realisation** of Research

