



Innovative Medicines Initiative

***European Induced  
Pluripotent Stem Cell  
Bank***

# Background

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- Researchers are looking for ways to validate pathways using human data. iPS cells are one approach to this.
- iPS research and banking is fragmented across a broad spectrum of institutions in the US and Europe
- There is limited access to quality patient derived iPS cells within a defined time line and full genotypic and phenotypic information
- There has been an exponential rise in interest in iPS cell research: access to such reagents now far outstrips their provision.
- Excellent opportunity (and time) to consolidate & strengthen the European Bioscience base and avoid duplicative effort
- Ideal way to maximise the use of funds, whilst maintaining costs, to benefit all



# Why IMI?

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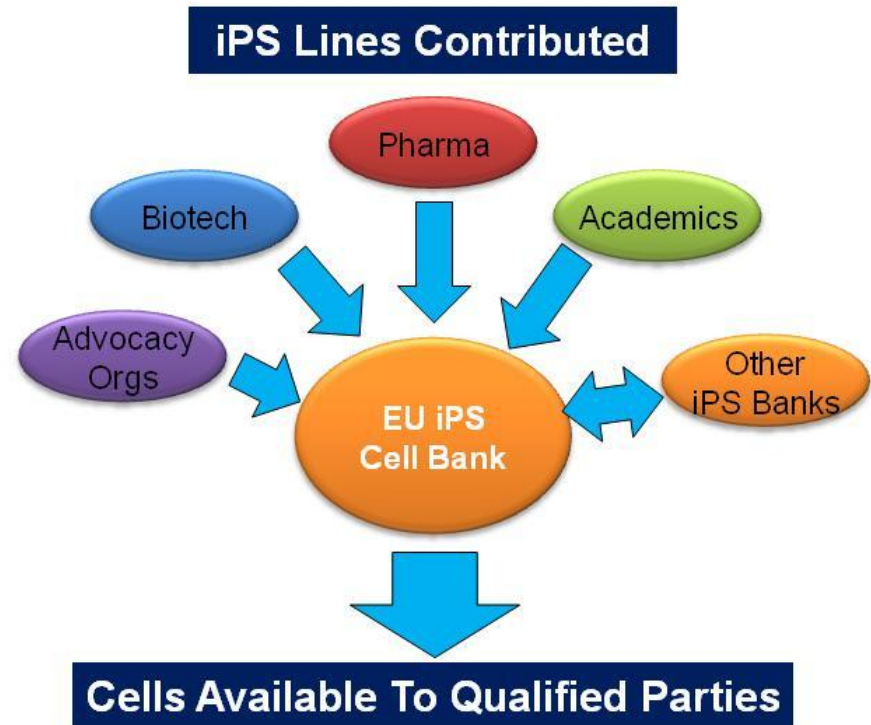
- There is a need for a coordinated approach to iPS cell access and standardisation across Europe
  - Unique opportunity to consolidate on-going IMI iPS cell projects across Europe and expand to include additional cohorts of iPS cells.
  - Ensure sustainable and easy access to iPS cell lines for Pharma biotech and academia
  - Unique opportunity to provide a first class European iPS cell banking facility
  - It will give a strong message that Europe, along with the US and Japan, is a major iPS cell centre of excellence
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# Need for public-private collaboration



- Rapidly increasing demand for efficacy and toxicity testing using iPS cells from disease relevant populations
- Linkage of disease properties back to the physiology of defined cells, as well as genetic linkage between patient and disease, would be an enormous step forward for drug discovery
- It will provide a centralised facility for academics, biotech's and pharma to store and retrieve their collections



An industrial scale, not-for-profit, storage and distribution centre for iPS cells across Europe



# Overall objectives



Identification of key cohorts of patients that are useful for research purposes within the wider scientific community

- Covers broad range of therapeutic areas/patient diseases
- Support academics who have key cohorts to generate the full complement of pheno or genotypic data (or missing iPS cells)
- Upon completion iPS cells banked in a central repository - open access to all

A large single European iPS cell repository in an appropriate facility

- Sustainable supply at low cost for IMI consortium members, academics, biotech's, and patient advocacy groups
- Consistent, high quality provision of iPS cells in a defined time frame to the bioscience sector
- Partnership with key iPS banks around the world to create a consistent approach to banking
- Strengthening of the European Bioscience base

Centre of scientific excellence for standardisation & optimisation in cryopreservation, retrieval & differentiation methods for iPS cell lines

- Standardisation of methodologies for generating iPS lines and/or differentiation protocols
- Provision of laboratory space and training facilities in stem cell culture



# Pre-competitive nature

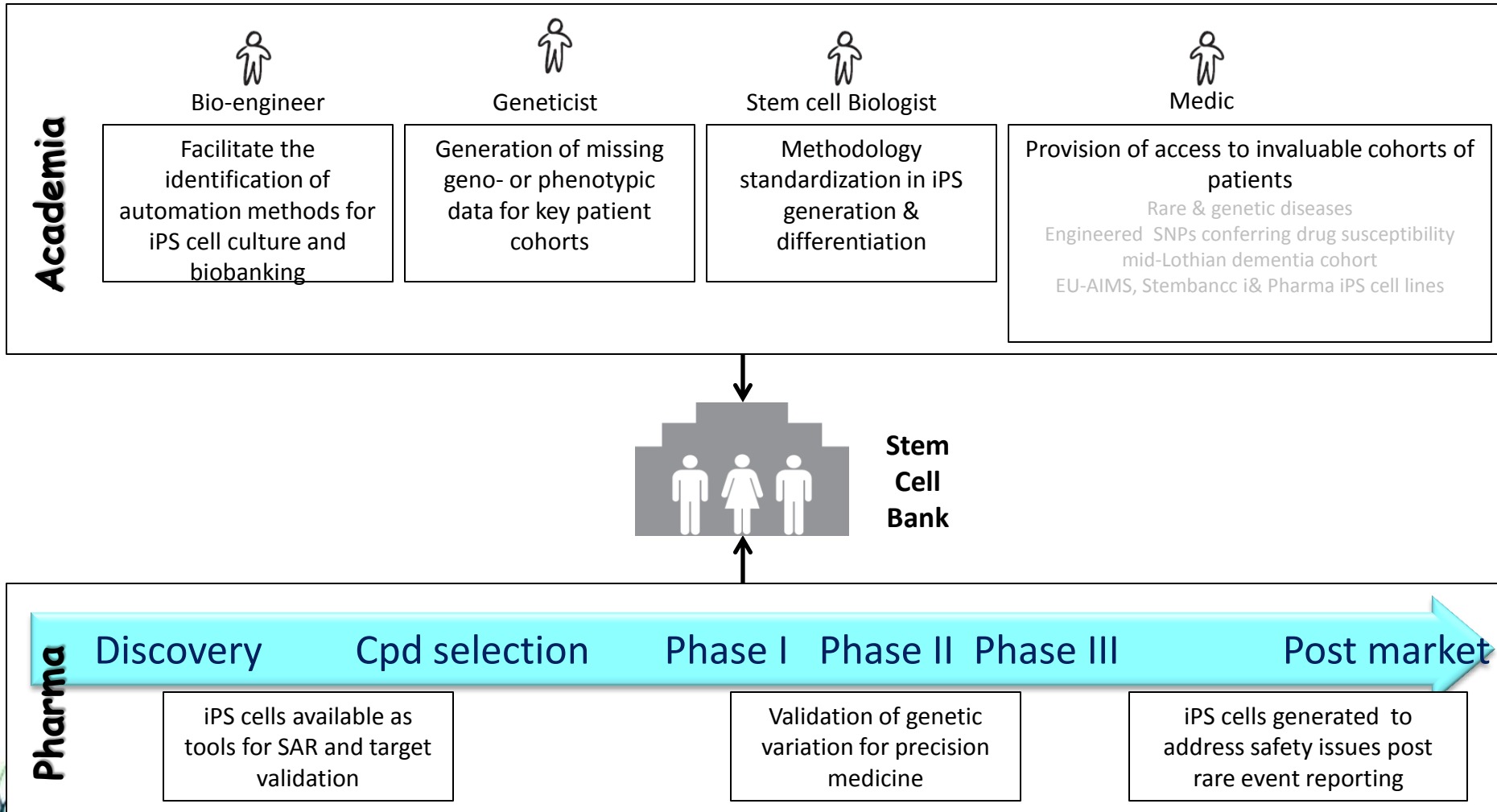
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- iPS cells are provided at early stages of the drug discovery process and can also enable patient stratification
- Opportunity to remove the burden of cell banking from publically funded iPS cell projects through the creation of a centralised repository for all existing European consortium providing iPS cells, such as:
  - FP7: SEURAT-1 & SC4SM
  - IMI: EU-AIMS & Stembancc
- Maximise availability of patient derived iPS lines for medical research
- Set up as a not-for-profit organisation: aim is to ensure costs are contained and an affordable pricing structure enables access to iPS cells for all scientists
- Unique opportunity to enable current biobanks, SME's, NGO's to work together to achieve a high quality enabling resource centre



# Expected impact on the R&D process



# Suggested architecture of the project

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- **Work Package 1: Governance/Policy**
  - A central governing body (akin to a board of directors) will be developed to oversee and coordinate the strategy as well as all activities of the iPS bank to create a sustainable business model
- **Work Package 2: Sustainability of iPS Lines**
  - Partnership with on-going European funded stem cell initiatives (e.g., STEMBANCC)
  - The inclusion of patient derived iPS cell lines that cover a breadth of disease areas
- **Work Package 3: Maximising iPS Collections**
  - Provide access to a wide and diverse range of patient derived iPS cell lines
  - The bank will remain abreast of the emergent reprogramming technologies, adopting those accepted by the wider scientific community
- **Work Package 4: Bio-engineering and Automation**
  - There is a clear need for the bank to facilitate the identification and implementation of automation methods for iPS cell culture and biobanking
- **Work Package 5: Ethics, IP & Training Considerations**
  - The bank will be responsible for ensuring the human tissue/somatic cells are acquired ethically and supplied in accordance with customer companies' corporate policies
  - iPS cells received by the bank are deposited with acceptable IP constraints, including reach-through rights
  - The iPS cell bank should provide facilities for training/education workshops on stem cell biology & cryopreservation that can be run independently or in partnership with institutions such as UKSCB and ECACC
- **Work Package 6: Banking Infrastructure and Management Systems**
  - Provide rigorous, standardized, scalable processes with clear documentation for cell freezing, revival and continued culture
  - The bank will provide an IT infrastructure that has the capability to link large data sets of information associated with each iPS cell vial in a way that complies with EU legal and ethical requirements





# Expectations from the Applicant consortium

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- **Generation of a business & operating plan for a pan-EU iPS cell repository that provides:**
  - Simple access to a wider & more diverse range of patient derived iPS cell lines, with associated genotypic, phenotypic and clinical data
  - Identification of an appropriate site(s) for the repository
  - Provision and maintenance of iPS cell tools for discovery research and precision medicine
  - Adherence to International Stem cell Guidelines
  - Consolidates knowledge on different iPS cell lines & the provision of iPS cell protocols and training
  - A reduction in cell banking infrastructure costs for subsequent IMI calls (removal of duplication)
  - Sustainability for IMI iPS cell lines from previous and future calls either pre or post the end of each funded project
  - An opportunity to seed new scientific advances as a result of the learning's gained from the provision of iPS cells



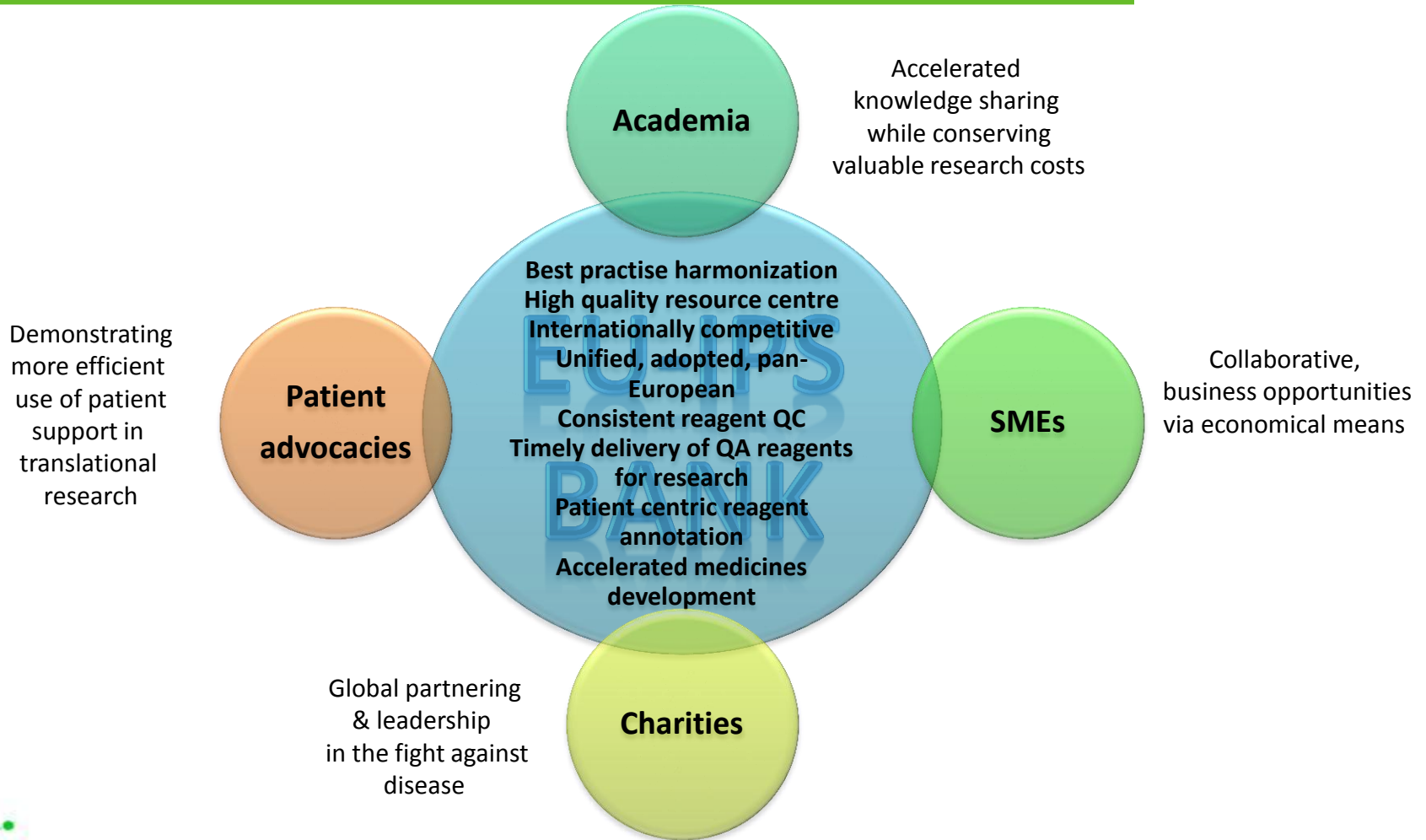
# Expected (in kind) contributions of EFPIA members



Current EFPIA members on-board	Suggested contributions
<ul style="list-style-type: none"><li>• Pfizer (coordinator)</li><li>• Sanofi</li><li>• NovoNordisk</li><li>• Servier</li><li>• Lundbeck</li><li>• Astra Zeneca</li><li>• UCB</li><li>• Others pending</li></ul>	<ul style="list-style-type: none"><li>• Business model &amp; financial expertise</li><li>• Legal and patenting expertise</li><li>• Scientific background on disease biology and expertise in stem cell biology and clinical practice</li><li>• Experimental support (FTE costs) for the generation &amp; characterisation of iPS cell lines &amp; missing genotypic or phenotypic data</li><li>• Contribution of iPS lines made by companies during the call</li><li>• Accessing of iPS cells from the bank</li><li>• Cash</li></ul>



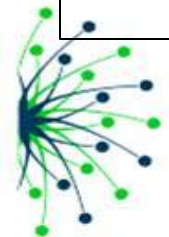
# What's in it for you?



# Sustainability for previous IMI iPS cell calls



<b>Advantage Of A Centralised Bank</b>	<b>Previous IMI Calls &amp; Other Initiatives</b>
<ul style="list-style-type: none"><li>• The iPS cell bank provides a sustainable model for IMI iPS cell lines from past and previous calls either pre or post the end of each funded project</li><li>• It would remove the burden of cell banking from these publically funded iPS cell projects through the creation of a centralized repository</li><li>• This should lead to a reduction in cell banking infrastructure costs for subsequent IMI calls</li></ul>	<p><b>IMI Call 4 – StemBANNC</b></p> <ul style="list-style-type: none"><li>• Stem cells for Biological Assays of Novel drugs &amp; predictive toxicology</li></ul> <p><b>IMI Call 3 – MIP-DILI</b></p> <ul style="list-style-type: none"><li>• Mechanism-Based Integrated Systems for the Prediction of Drug-Induced Liver Injury</li></ul> <p><b>IMI Call 3 – EU-AIMS</b></p> <ul style="list-style-type: none"><li>• European Autism Interventions – A Multicentre Study for Developing New Medications</li></ul> <p><b>BBMRI-ERIC</b></p> <ul style="list-style-type: none"><li>• Opportunities for integration of the bank into BBMRI-ERIC should be explored</li></ul>



# Key deliverables of full project

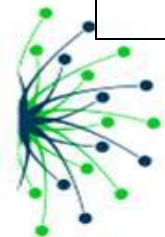


## Scientific

- Generation of a full complement of geno- & phenotypic data for key patient cohorts that would be useful to the scientific community
- Research & implementation of current gold standard best practises for the generation and differentiation of iPS cells including qualified differentiation cocktails, qualified culturing conditions
- Development of automatable processes for iPS cell culture and banking
- Application of best practise in cryopreservation & biobanking in order to develop a 'commercial standard' state of the art iPS facility
- Provision of quality protocols and training in iPS cell growth & development

## Operational

- Set-up of a sustainable, not-for-profit, specialist production, storage and distribution centre for iPS cells across Europe in an appropriate location
- Provide patient derived iPS cells to a defined quality and within a defined time from placing an order
- Supply an iPS differentiation service during the latter half of the call
- Provide searchable anonymized geno-, phenotypic & clinical data associated with each iPSC line



# Questions?

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- Contact the **IMI Executive Office**

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Website: [www.imi.europa.eu](http://www.imi.europa.eu)

