

# Executive Summary

## Period 1 (January-December 2014)



### *Project rationale and overall objectives of the project*

The European Bank for Induced Pluripotent Stem cells (EBiSC) Consortium will establish a facility for distributing qualified human, disease representative stem cell lines for research. The EBiSC cell line repository will make future drug development more effective and provide resources for future EU-funded iPSC projects.

Key objectives of EBiSC are to establish a single European iPSC repository with unique identifying features of a catalogue created by user demand. Provide sustainable supply of quality assured, research grade lines on a not for profit basis. It will develop procedures for engaging a wide scientific and clinical community in a network of cell line derivation centres. It will apply scientific excellence for standardisation of optimised methodologies for deriving iPSC, their cryopreservation, recovery and differentiation. It will demonstrate standards in quality control for the routine banking, characterisation and distribution of cell lines. The cell line distribution model will be supported by a harmonized ethics and legal governance framework and information management system developed to accommodate user-generated content. It will establish mechanisms to facilitate ongoing stakeholder enhancement of the biobanking process in support of a strategic business strategy based on a phased execution to ensure self-sustainability.

### *Overall deliverables of the project*

Demonstrating effective project management, to include both strong financial and strategic leadership, developing the banking business rationale tuned to user's needs and linking this to an EBiSC brand are key deliverables from overall project development. A key consequence of developing a better understanding of user needs will lead to the recruitment of additional EPFIA companies as integrated partners.

Procuring cell lines that researchers will use to constitute the diversity collection and demonstrating effective infrastructure for centralized processing of these, storage and international distribution by harmonized protocols, are core deliverables from the bank operations. Phenotypic assay data from the use of selected cell lines from the collection will ensure that the project delivers validation on all elements of the cell line supply chain.

The project will provide deliverables that reflect a movement beyond current art status in platforms for improved cell processing, cell line QC testing, information management and innovation in human cell line banking governance models. Deliverables related to the development of an EBiSC cell line collection that researchers are using and operations for engagement with paying customers will be key for future self-sustaining business.

### *Summary of progress versus plan since last period*

**The EBiSC iPSC Catalogue** The Hot Start collection has 38 processed iPSC lines available at M12.

This is 30% fewer than anticipated due to a combination of factors concerning nominated existing lines such as a delayed start to cell expansion (partner 26: ISCI), failure to comply with EBiSC SOPs for cell vial preparation (partner 13: KNAW), lack of ethical clearance to contribute lines into the collection (partner 18: BiP) and unresolved embargo on sharing iPSC lines (partner 9: UNEW). Twenty eight (28) cell lines were banked from the remaining public partners as planned for the Hot Start. The EBiSC project has nominated 500 lines from the Sanger hiPSCi project, for the preparation of vials for distribution by EBiSC. These have yet to be processed. Two factors have contributed to this delay; firstly a pre-existing arrangement for distribution of hiPSCi lines from the Wellcome Trust Sanger Centre and ECACC (partner 17a: DH-CC) needed to be fulfilled and a delay in EBiSC dedicated staff recruitment by partner 5 (Sanger) led to a capacity restriction for preparing the vials for distribution as part of the EBiSC Foundational Collection. Pleasingly, an unexpected allocation of ten (10), third party lines (partner 15: UCL) were deposited and processed by the Central Facility.

The Consortium Board (CB) has approved two new line commissioning project proposals (approximately 50 future novel patient cell lines), total costs €503,120 of which EFPIA contributions are €125,780 (*see section **Error! Reference source not found.** of the present report and appendix X of the DoW for details*). These proposals were instigated in direct response to EFPIA partner interests in accessing novel lines. EBiSC has identified a large number of existing EU funded iPSC generation projects, with conservative estimates that the catalogue can be augmented by an additional 2,500 distinct patient derived lines.

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### Work-package highlights underpinning EBiSC workflow

Derivation centre wide adoption of Standard Operating Procedures (SOPs) for the central workflow has occurred. SOPs for the shipping of bio-samples have been developed. An international standard cell line Quality Control (QC) platform is operating with determination of cell line identity, plus also both gene and protein marker expression confirmation of pluripotency. Non-integrating methodologies are being used for *de novo* derivation.

Unique data sets from collaborative disease modelling using Hot Start lines are now compiling after a delay, as cell line distribution to EFPIA partners did not commence until quarter 4 of period 1 (P1).

SOPs for bio-sample procurement, sample tracking remain in development. Although the information management system design has been specified, its construction is delayed and so inter-operability with the central processing facility LIMS, cell line registry to support sample tracking is untested. Steps are being taken to correct this delay to enable the prototype cell line tracking with the IMS by month 18.

### Work-package highlights underpinning EBiSC business strategy

Harmonized principles for the consenting of patients for bio-sample donation and agreements for cell line deposition into and third party access and use from the EBiSC catalogue have been adopted. An initial survey of the research community interests in and requirements for qualified iPSC lines generated data has been performed for informed decision making for designing the 'Hot Start' collection and contributing to the white paper on the EBiSC vision and business strategy. An Freedom-to-operate (FTO) analysis has been carried out (D1.1.2), but delivery of the IP Strategy is delayed pending further business analysis. Version 1.0 of the business plan has been delivered.

### Progress towards meeting planned Deliverables & Milestones

Of the 24 deliverable reports due in the first reporting period (P1), all are now complete and ready for submission. Of the 34 project milestones due in P1, one (M1.1.7) remains open pending further development of the business strategy and will be carried into reporting period 2, three (M1.1.3, M6.1.2, M4.1.4) are in progress pending completion in quarter 1 of 2015 and the remaining 30 milestones are all complete.

### New project partners

Bayer Pharma AG have expressed interest in joining the consortium and confidential discussions are advancing on how they will contribute to the project.

### Significant achievements since last report

Executive Office has prepared V1.0 of the Strategic plan. Reference iPSC have been used to test and validate protocols for the whole workflow, tracking and transport. The first cohort of Hot Start cell lines has been delivered to DH-CC. Donor consent forms, harmonized deposition and user/access agreements have been deployed. The central processing facility (partner 3: RC) has successfully distributed a number of lines to partners and third party users, demonstrating impact delivery to the research community in particular EFPIA. Gap analysis of what EFPIA partners need and the lack of unencumbered, appropriate existing cell lines, triggered commissioning of new lines ahead of schedule. New operational laboratories at EBiSC central processing facility (Babraham research facility) was taken ahead of schedule. A successful pilot study was undertaken in collaboration with the IMI project StemBANCC.

#### Information on EBiSC

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#### Access to the EBiSC iPSC Catalogue

<https://cells.ebisc.org>

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