

ANNEX A

DIFFERENCES BETWEEN STDR PRE-PILOT (STREAM 1&2) AND PILOT STAGES

The **Pre-Pilot** stage aims to support earlier-stage and more exploratory, proof-of-concept projects with some preliminary data. Areas supported include the preclinical validation of drug targets, or platform exploration.

The **Pilot** stage aims to support more mature projects that have more data and have already been validated to a certain degree, or already have a commercialisation plan.

Comparisons between Pre-Pilot and Pilot

<u>Pre-Pilot Stream 1 (Target Validation)</u>	<u>Pre-Pilot Stream 2 (Platform Development)</u>
<p>Stage: Preliminary data supporting the implication of a target in a given disease indication, with new data generated by the applicant and/or key published experiments reproduced by the applicant.</p> <p>The target should have a novel role in the indication chosen by the applicant.</p> <p>Note: Large scale compound screening, Hit-to-Lead or Lead-optimisation cannot be supported by the Pre-Pilot Stream 1.</p> <p>Examples of projects:</p> <ul style="list-style-type: none"> • Validation of XXX as a relevant target against xyz indications • Peptides as tool inhibitors of XXX for xyz indications • Novel Biomarker and therapeutic target in xyz indications • Validating the tractability of XXX as a target for antibody therapeutics <p>Mentorship/training: A Drug Discovery Specialist (DDS) will be appointed to each project to work with the investigator on the design of key experiments needed for preclinical target validation. Projects will attend a bootcamp organised by EDDC to better understand considerations for developing therapeutic assets for commercialisation.</p>	<p>Stage: Preliminary data supporting the proof of concept for a platform technology.</p> <p>An indication of the scope of applications but no specific set of key commercial applications defined.</p> <p>Definition of platforms: Platforms are technologies that enable broad application of the underlying science and in so doing, create value by enabling multiple applications or products.</p> <p>Examples of projects:</p> <ul style="list-style-type: none"> • The invention of CRISPR technology for genome editing as enabling broader applications of genetic engineering to meet unmet medical needs • Generalisable combinatorial screening platform for unbiased interaction discovery • Microfluidics for High-throughput Antibody discovery against XXX • XXX cell-derived cancer immunotherapy platform <p>Mentorship/training: Each project will be assigned a mentor trained by SMART using the Adaptive Innovation™ Framework. Projects will be supported with a venture exploration bootcamp organised by SMART that will help to scope out the key potential applications with commercial impact and help outline the commercialisation pathway.</p>



Intended outcome: Target validation, more convincing evidence of whether the target should be considered for a drug discovery campaign.

Intended outcome: Validation of the platform and a clearer commercialisation pathway.

Pilot

Stage: Funds more mature projects that have already been validated to a certain degree. Both single target/single asset projects and platform technologies projects will be funded, in a range of stages from target validation to preclinical work.

For single-asset projects: Data demonstrating that

- i. The target is implicated in the disease indication;
- ii. The target can be modulated therapeutically;
- iii. Modulation of the target may lead to a therapeutic effect in relevant pre-clinical models; and/or
- iv. A defined path towards the development of a therapeutic candidate.

Example workplan for single-asset Pilot projects:

- Phase 1 – Include the relevant activities such as identification of target modulators (e.g. high-throughput screening), lead generation, preliminary evaluation of efficacy and safety of candidates in-vitro and in-vivo, development of in-vivo models for preclinical assessment.
- Phase 2 – Optimisation of lead candidates, development planning, initiating assembly of data package for preclinical candidate

For platform technologies:

Data supporting and establishing the platform technology, such as early data showing two or more applications, and a clear pathway articulated to select and demonstrate a first commercial product.

Example workplan for platform technologies Pilot projects:

- Phase 1. Successful in vitro killing of cancer cell lines with engineered iPSCs out of the platform technology.
- Phase 2: In vivo assessment of survival in cancer mouse models.

Mentorship/training: Pilot awardees will attend mentoring sessions to receive advice and support from leading entrepreneurs and innovators (including VCs, ex-pharma, biotech CEOs, or experts from EDDC). Mentors will provide advice on matters relating to the development and commercial strategy, including marketing, business modelling and pitch practice/advice.

Selected awardees will attend STDR bootcamps organised by SMART that will help clarify the key commercial applications and define a development pathway.

Intended outcome: Allow projects to have sufficient funding to bridge the gap to the next value inflexion point in the drug discovery and development pathway. Outcomes include follow-on funding from private funders, co-development with industry or entry into EDDC's portfolio.