**Modulo di descrizione del Progetto**

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| **Project Title** |  | **Target Gene Name**  *e.g. PDCD1, JAK3* |  |
| **PI name** |  | **Target Gene ID**  *e.g. 5133, 3718* |  |
| **PI email** |  | **Disease Area**  *e.g. Oncology, neurology, immunology* |  |
| **Institution Project Number** |  | **Mode of action**  *e.g. agonist, antagonist, inhibitor* |  |
| **TTO Contact** |  | **Modality**  *e.g. small molecule, antibody, gene therapy* |  |

|  | **Brief summary of evidence** | **Evidence evaluation (ideal/acceptable/undesirable)** |
| --- | --- | --- |
| **What do we know about the target biology?** | |  |
| 1. Do we have HUMA/BACTERIAL/VIRUS genetics data associated with functional LF/GF/Dom Neg etc.? |  |  |
| 1. Are there any close homologues of the target that might impede selectivity? |  |  |
| 1. Detail the existing evidence that demonstrates a link between the target and the disease |  |  |
| 1. What are the safety implications of modulating the target? (Activation or inhibition) |  |  |
| 1. How widely is the target expressed |  |  |
| 1. Is there a cellular proof of principle? |  |  |
| 1. Is there an *in vivo* proof of principle (also clinical data)? |  |  |
| 1. Is the underlying biology recapitulated in rodent models? |  |  |
| **Is the target druggable?** | |  |
| 1. Does the target have a well-defined small molecule binding pocket? |  |  |
| 1. Is the target exposed at the cell surface or secreted to allow interaction with an antibody? |  |  |
| 1. Are there any described reference compounds /antibodies/tools available to aid the development of an assay? |  |  |
| 1. Is the target structurally enabled? |  |  |
| 1. Describe which reagents and protocols you currently have available to aid drug discovery. How robust and reproducible are these? |  |  |
| **Do we have developable chemistry (applicable only for chemistry generated by the PI)** | |  |
| 1. Are the molecules in a desired physicochemical/drug like space? |  |  |
| 1. What is the activity against the target and related targets that may play a role? |  |  |
| 1. Is there any early DMPK data available (met- stab/solubility) |  |  |
| 1. What are the safety risks associated with the chemistry? (Structural alerts) |  |  |
| **What do we know about the future clinical use?** | |  |
| 1. Describe the principal clinical applications |  |  |
| 1. What is the intended patient population? (Disease condition, demography, co-morbidities) |  |  |
| 1. Describe the current standard of care, likely co-morbidity and clinical outcome. |  |  |
| 1. What is the intrinsic variability of the target in the population? (Stratification) |  |  |
| 1. What is the competitive advantage of the proposed target/approach compared to existing therapies and those in development? |  |  |
| 1. What is the preferred route of administration? |  |  |
| 1. How large is the market in terms of patient population? (Orphan/small/medium/large) |  |  |