CD Laboratory with an international R&D partner

Immune Therapy-mediated CAncer REjection via NR2F6 blockade

Acronym: I-CARE

Gottfried Baier and Team
Medical University of Innsbruck

Global „Cancer Burden“

Finding ways to boost host-protective cancer immunity
TARGETING THE IMMUNE CHECKPOINT PATHWAY IN IMMUNE CELLS
(e.g. via PD-1/PD-L1 or CTLA-4 blocking biologicals)

Immunotherapy demonstrates extraordinary rates of long-lasting responses for a variety of the most difficult-to-treat cancers.

However, there remains an unmet medical need, as still only a limited number of patients respond to cancer immunotherapy regimens today.
Concept of TaNeDS (Take a New Challenge for Drug Discovery)

Research stages:
- Idea & Exploratory Research
- Discovery of technologies/target molecules
- Validation of technologies/target molecules
- Establishment of concepts of invention
- Enhancement of exclusive effects of patents
- Working toward practical use for proprietary technologies

Extended Collaboration:
- Type A Collaboration:
  - Type A: €50,000 – 80,000 per year, plus overhead x 2 years
- Type B Collaboration:
  - Type B: €100,000 – 150,000 per year, plus overhead x 2 years

As such a BigPharma industry partner capable of performing an in-house compound development program is not available in Austria, we choose DS for our high-end R&D project.
Preclinical investigations

NR2F6 – Academic focus

NR2F6 – Translational focus

Associated academic and clinical collaborations

Daiichi Sankyo-based small molecule NR2F6 checkpoint blockade drug finding program

These inter-related I-CARE research objectives will ensure a high probability of yielding both conceptual, technological and translational synergies, ultimately allowing us to evolve into an internationally-visible CD laboratory for cancer immune therapy!
Synergy Effects and Long-term Perspective:

„Bridging the gap between basic immuno-oncology research and the cancer patient’s bedside“

I-CARE mission statement:

Exploiting our academic knowledge of the NR2F6 signaling pathway that governs pathological cancer immune-evasion for the development of a NR2F6-based therapy

- Unique Austrian joint venture of academia & industry committed to translational immuno-oncology research: Interdisciplinary R&D research comprising BigPharma chemists, molecular immunologists, bioinformaticians and clinicians with streamlined translation of the common research plan, with sharing of expertise and research tools and with access to enabling technologies & data warehouses

- Advantage for the Austrian University: International visibility, collaboration with BigPharma and creation of career opportunities; R&D output as high-impact publications

- Advantage for the industrial partner DS: R&D output as patents and products

Product development and all clinical studies itself will be exclusively performed by DS and these tasks are clearly excluded from our proposed CDL I-CARE work in Innsbruck!
Structure of communication, cooperation, and information for decision-making

- **Objective 1 & 2 teams**
  - every 3 months
  - every months

- **Work package teams**

- **Subtopics**

- **Board Meetings (WebEx)**
  - twice a year

- **Strategy Meetings**
  - (in-person; alternating in Innsbruck or Tokyo)
  - once a year

- **CDL Management**
  - Objective 1
  - Objective 2
  - Collaborations
  - DS-based R&D

- **CDL I-CARE INITIATIVE**

- **Collaborations**
  - Finance
  - IPR
  - Publication
  - PR

- **DS-based R&D**

- **Associated academic and clinical collaborations**

- **Daiichi Sankyo-based small molecule NR2F6 checkpoint blockade drug finding program**

- **Associated academic and clinical collaborations**
CDL for Cancer Immune Therapy in Innsbruck

MUI-Clinic

Dep. for Pharmacology and Genetics
incl. CDL I-CARE ?

Daiichi Sankyo-based small molecule NR2F6 checkpoint blockade drug finding program

Associated academic and clinical collaborations

NR2F6 - Academic focus
NR2F6 - Translational focus

CDL CARE INITIATIVE

NR2F6 - Academic focus
NR2F6 - Translational focus

Associated academic and clinical collaborations

Dep. for Pharmacology and Genetics incl. CDL I-CARE !?
Key personnel proposed for I-CARE

-------------------------------

**CDL-funded personnel:**

1 postdoctoral position (24 PM/2a) for the project-experienced Dr. Victoria Klepsch
   2 PhD students (48 PM/2a)
   2 diploma students (24 PM/2a)

**In kind-funded personnel:**

PI Gottfried Baier (40% commitment = 20 PM/2a)
   1 technician (12 PM/2a)
   plus extra management and animal care personnel

------------------------------

Total of 128 PM in the first 2 years

The scope and the vast majority of the work described in this proposal is appropriate for the requested (wo)man power!
During the last 15 years, G. Baier contributed to the "Oncoscience and Immunity Clusters" and the campus development at the Medical University of Innsbruck.

- **Systems biology project “SYBILLA”**, USA-based

- **ONCOTYROL** “Center for personalized cancer therapy”, European Academy of Tumor Immunology

- **Doctoral College MCBO**: “Molecular cell biology & oncology”

- **SFB021**: “Cell proliferation and cell death in tumours”
During the last 20 years, Gottfried Baier contributed to the „Oncoscience and Immunity Clusters” and the campus development at the Medical University of Innsbruck.

Next Task: Establish an internationally visible “CD Laboratory for Cancer Immune Therapy” that is built on our longstanding basic and clinical expertise in Innsbruck!
Christian Doppler Labor
für Krebsimmuntherapie mit
pharmakologischem NR2F6 Inhibitor

Module 1 start: Dec 1st, 2016; Research budget of € 2.200.000,--