European infrastructure
for translational medicine

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Presentation subject

Translational medicine advice given under CORBEL

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NATURE OF THE SERVICE

- Help Desk advice on regulatory matters relevant for product development
- Part of feasibility advice
- Relatively small number of requests (but growing)
- From both academia and industry (SMEs)
- Sustainability post-CORBEL through 2 part time consultants

Innovation Tool Box - Procedure

Template 1
- Identification of the user
- Non confidential description of the request

Day 0 - 2.

Template 2 (after CDA)
- Confidential data and questions / request
- Experts identification
- Fill in with answers / evaluation

Day 3 – 60

Written Report
- Question's answers
- Service identification

Day 60

Question's scope

- Translational development plan – issues and progression
  - Scientific Advice
- Regulatory authority approach
  - Data certification
  - Tools qualification
  - Orphan drug designation
  - Marketing and clinical trial Authorisation
    - IMPD
    - IB
    - MAA
- Post Marketing and reimbursement approaches

Access request received

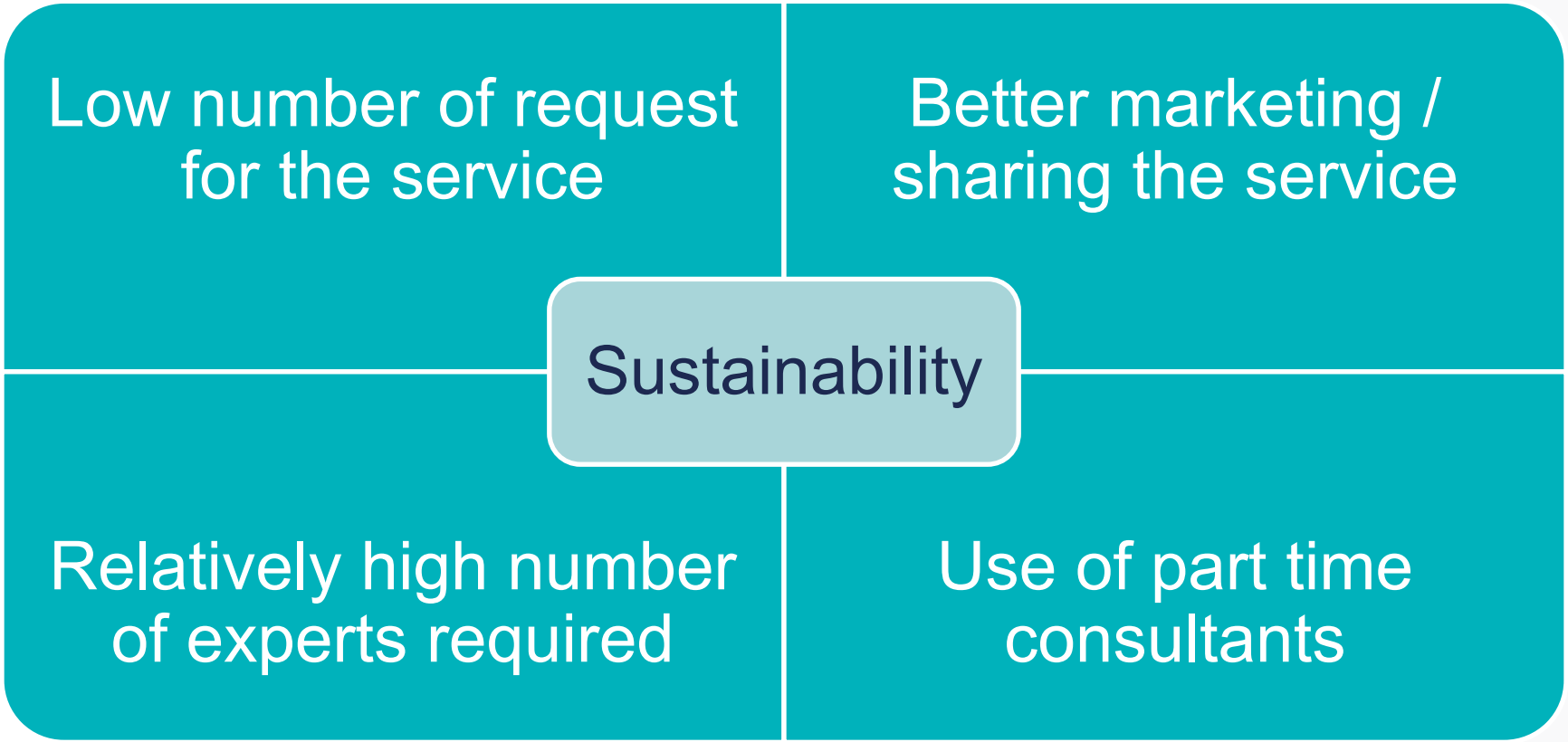
- Ten request of access from Academic and SME in three years 2017-2020
- Time employed to close the request ranging from 2 months to 14 months
- Effort required by the team from 1 week to three months of a FTE

Why such a range in time and effort ?

- Quality of the questions
  - Specific question about the admissibility of a specific product to receive incentives were easily answered
  - General question about the product development required more time as often the identity of the product itself was not yet defined
  - Approach to Competent Authority were the longest due to the necessity to translate academic language into specific regulatory questions. Even a simple scientific advice required training for the investigator to correctly define their question

FOUR CASES UNDER CORBEL

- Treatment for rare inherited disease. A request to assess the market potential and routing for a repurposed drug applied to a rare disease. Required two months to analyse and prepare the answer.
- Small company with an innovative product to facilitate the Blood Brain Barrier crossing. Requested an assessment of the non clinical data and of the pathway to marketing. One month for a preliminary discussion of the product characteristic, classification and design of further steps.
- Stem cell based therapy (SME) A request of the non clinical data assessment in function of marketing and clinical trial authorisation. One month required for the assessment and prepare the answer. Items of discussion were the classification and the orphan drug status.
- A consortium of academics applying for funding a clinical trial with a compound treating Leishmania infection. Six months required to prepare and discuss a non clinical plan leading to a Clinical Trial Authorization. Identification of quality and safety items required.


Low number of request for the service
Better marketing / sharing the service
Sustainability
Relatively high number of experts required
Use of part time consultants

- Thanks for your attention