Webinar | IMI2 - Call 9 ‘Development of immune tolerance therapies for the treatment of rheumatic diseases’

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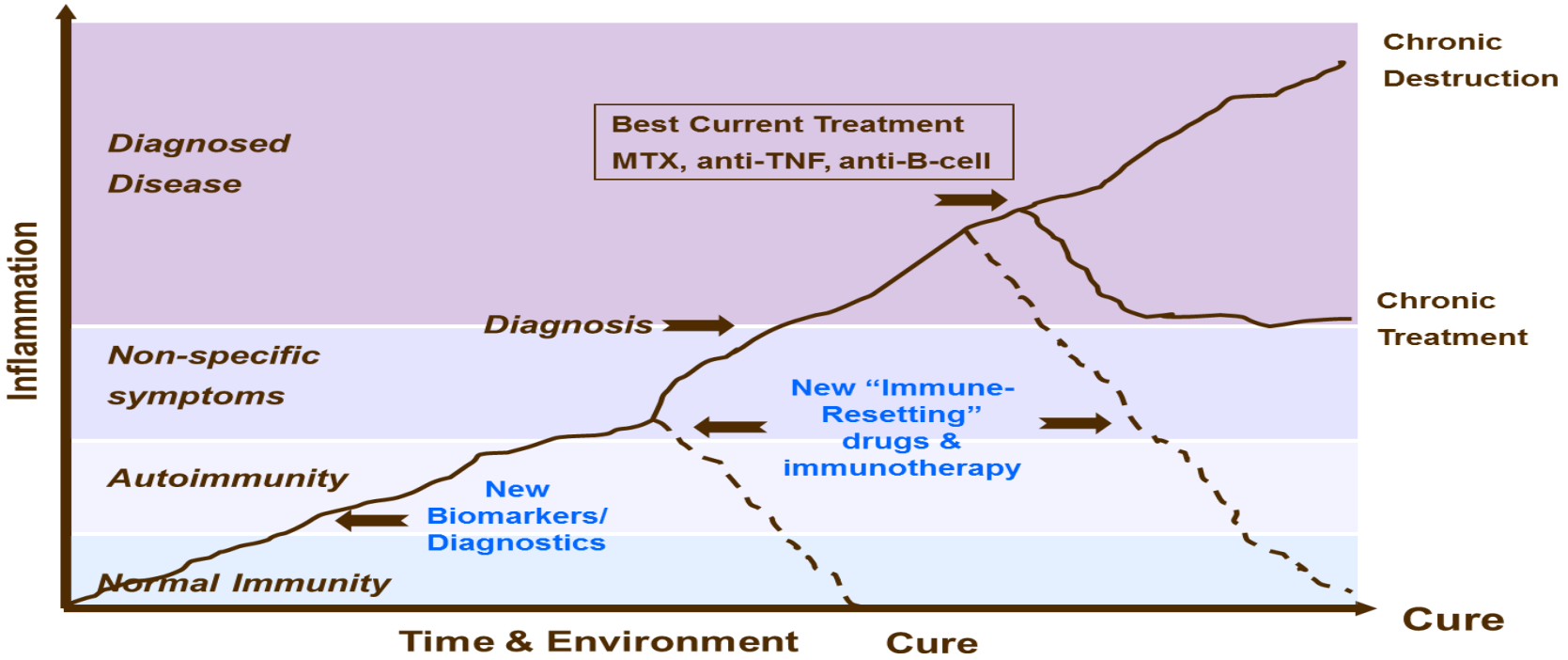
Potential Impact for Patients



- Established RA - tolerance therapy could provide drug free remission for patients.
- Prevention of RA - Screening initiatives with diagnostic tests to identify "at-risk" individuals.
  - Treatment of pre-arthritic patients may re-set and stop the immune-driven disease processes before inflammation & joint damage has occurred.
- Offers the healthy but "at-risk" patients a screening and "vaccination-like" treatment for the prevention of RA.

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Need for public-private collaboration

- Drug free remission through immune tolerance is the “Holy Grail” of immunology research. Novel therapies are required.
- Collaborative efforts in public private partnership are most likely to achieve this ambitious goal.
- Critical need for biomarkers to allow:
  - Patient stratification – select patients most likely to respond
  - Detection of immune-tolerising effects in patients during early clinical trials
  - Interventional clinical studies to explore tolerance potential of existing drugs in the right patient population.
- Requires extensive collaboration:
  - Patients & Clinical investigators, Biopharma industry & SME’s, Patient Groups & Regulators

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Objectives of the full project

- Development of tools for immuno-monitoring of T and B cells and other immune & inflammatory cells.
- Identification of relevant RA patient cohorts that would be suitable for testing “tolerising” therapies.
- Patient selection could be based on genetics, response to therapy, immune phenotype and auto-antibody profiles and others.
- Conduct experimental medicine studies using a small number of highly selected RA patients to monitor changes in immunity following therapeutic intervention (immunomodulators, peptide immunotherapy and other approaches).

Pre-competitive nature

- Building on the outcomes of the existing PP initiatives is desirable
- BT-CURE
- RA-MAP
- Tolerance Restoration In Auto-immune Diseases (TRIAD).
- The NIH “Tolerance Network” as well as the newly started “Accelerated Medicines Initiative” would be key collaborators.
- Others

Expected impact on the R&D process

- Improved scientific understanding of the regulation of immunity in RA may be transferable to other autoimmune diseases (eg. T1D, MS). Identification of novel drug targets & pathways

- Biomarkers that reflect a quiescent or “tolerant” immune state would enable short “proof of concept” studies to test future novel therapies.

- Patient Stratification - Ability to select the patients most likely to respond to “tolerising” therapies will reduce failure rates in clinical trials.

Suggested architecture of the project

- WP1: Project Management, Coordination, dissemination and sustainability - Overall coordination of the scientific work package delivery & budgets.
- WP2: Technologies for monitoring immune state - immuno-phenotyping of patient samples using state of the art technologies.
- WP3: Patients, Cohorts and Ethics - Analysis of retrospective samples, patient selection & coordination of new clinical studies.
- WP4: Mechanisms of immune tolerance basic research - analysis of patient tissues and samples to identify novel targets and pathways relevant to immune tolerance.
- WP5: Bioinformatics and data - Integration of historic and prospective data for the identification of biomarkers, stratification of patient cohorts and new drug target identification.

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Expected contributions of the applicants

- Experienced in the clinical investigation of RA. Particularly for the early treatment of pre-arthritic patient populations and biomarker qualification.
- Detailed understanding of immune response in RA and methods for immune monitoring.
- Access to patient cohorts, samples and the ability to conduct interventional studies.
- SMEs that can provide relevant immune monitoring technology.
- Involve patient groups and seek regulatory advice from EMEA and FDA as required

Expected (in kind) contributions of EFPIA members

- Provision of reagents including proteins, antibodies and small molecule tools.
- Provision of clinical samples for analysis.
- Access to cell analysis technology and "omic" technology.
- Bioinformatic analysis.
- Experimental therapeutics – drug substance.
- Post-doctoral funding.
- Health informatics tools providing direct contact between patients and healthcare professionals to study early stages of disease (pre-RA)

What's in it for you?

- Academic researchers
  - Opportunity to discover and deliver transformational therapies to patients
  - Improved understanding of immunity & tolerance in autoimmune disease
- SME's
  - Opportunity to develop and validate new technology in a clinical setting
- Patients' organisations
  - Valuable patient input on clinical un met need and the patients perspective

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Key deliverables of the full project

- Biomarkers for monitoring the development of autoimmunity and a tolerant immune state. This is a pre-requisite step in order to advance novel therapeutics into the clinic.
- Methods to stratify patients for clinical studies of immune tolerance. This is essential to reduce failure rates in early trials and deliver novel therapies to the right patients.
- Methods for T & B-cell phenotype and function and monitoring of autoantibody profiles and others
- Identification of new drug targets and pathways with the potential to induce immune tolerance
- A repository for new data will be established to allow mining for new targets and pathways.

Questions?

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