



UNDERSTANDING CHILDHOOD (CHRONIC) AILMENTS NETWORK - ASIA





AGENDA



UCAN-A

TRANSLATIONAL MEDICINE IN GLOBAL HEALTH

ONE EXAMPLE OF TECHNOLOGY FOR THE PEOPLE



WHAT IS TRANSLATIONAL MEDICINE?





UNMET MEDICAL NEEDS

Patients

Technologies

Does Technology reduce or broaden
the gap in Global Health?



EX PLURIBUS UNUM



UCAN-A IS MEANT TO BE A COORDINATED ECUMENICAL NETWORK WHERE ACCESS TO DATA, SAMPLES AND RESOURCES IS TRANSPARENTLY GRANTED BASED ON QUALITY OF SCIENCE AND LEVEL OF CONTRIBUTION

THE OBJECTIVES ARE NOT FAME OR FORTUNE, BUT TRANSFORMATION OF TRANSLATIONAL PEDIATRICS FOR THE BENEFIT OF PATIENTS AND SOCIETY

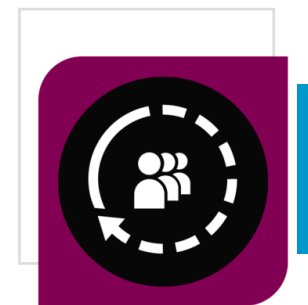


UNMET NEEDS



DELIVER STANDARD OF CARE

Dramatic need to deliver standard of care in many underserved areas



PATIENT PASSIVITY

Patients and their families are passive subjects both in care delivery and in translational research. They receive therapy and give biological samples without a true involvement and understanding.



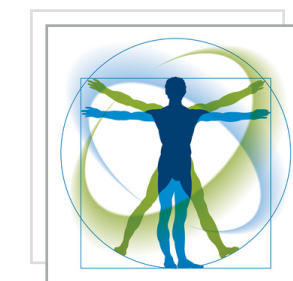
CULTURAL FRAGMENTATION

Cultural fragmentation. Lack of critical mass of patients for meaningful studies.



UNITE CLINICS & RESEARCH

There is a need to shorten the distance between state of art clinical care and translational research, in order to make research impactful and meaningful to society

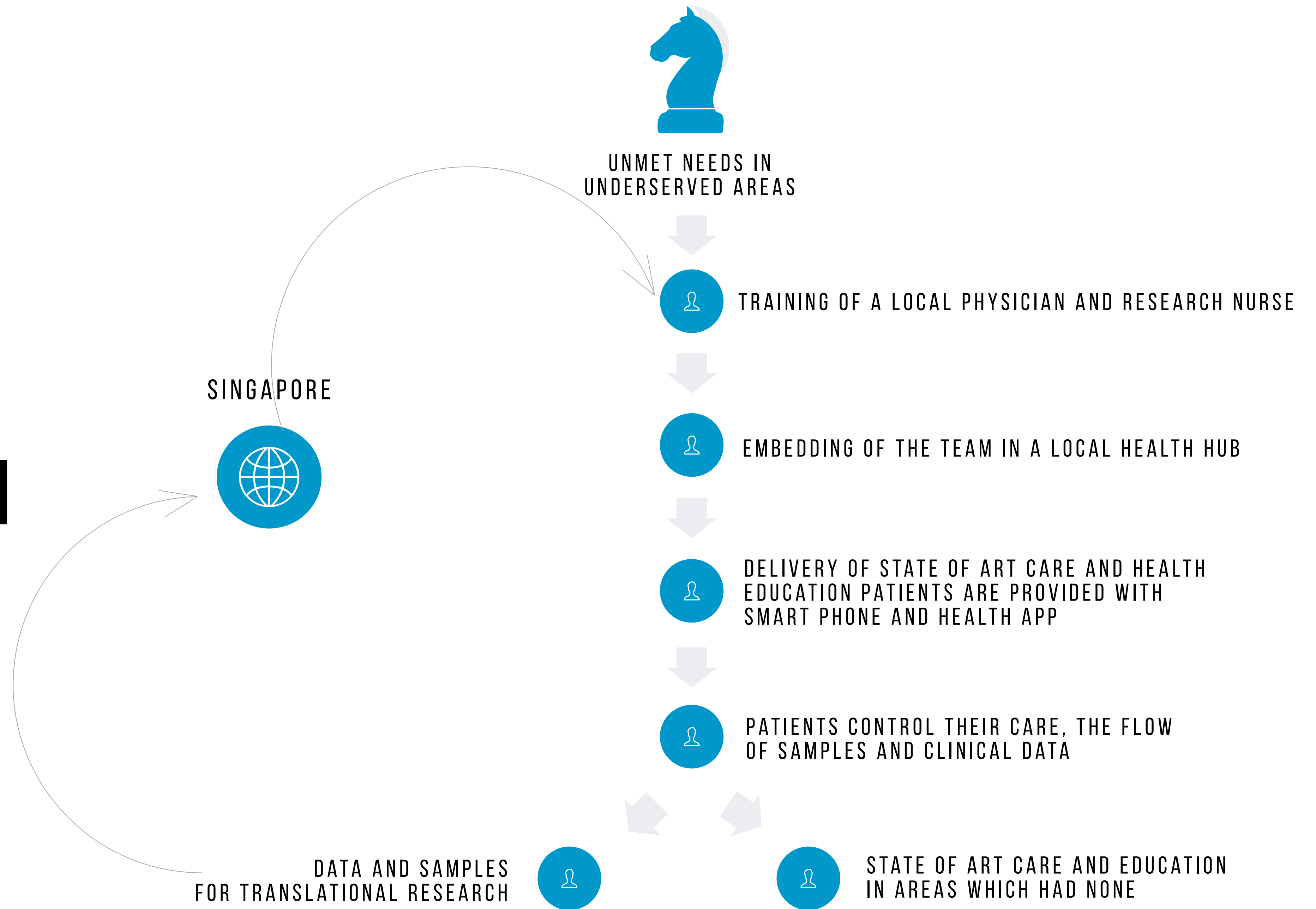


MOLECULAR GENOMICS KNOWLEDGE

Lack of adequate research infrastructure to cover the full spectrum of 'Omics" to unravel the molecular and functional genomics knowledge needed to better diagnose and treat such diseases.

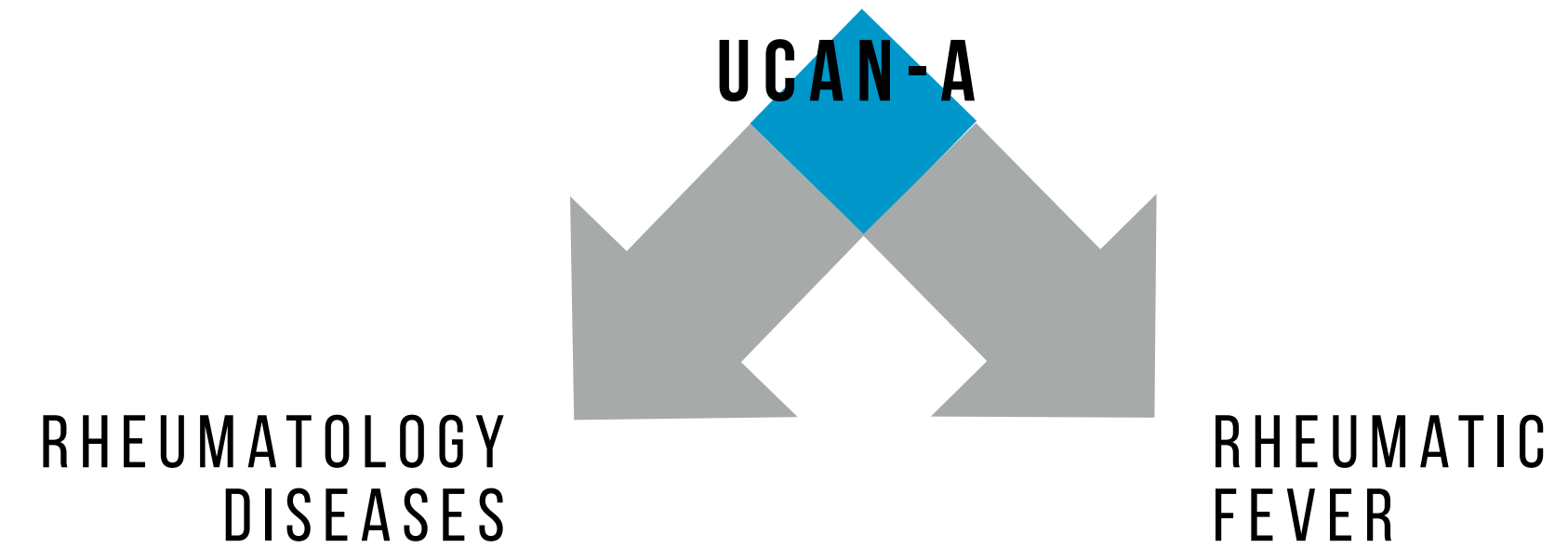


UCAN-A APPROACH





CHALLENGES AND OPPORTUNITIES FOR PHASE 1



- State of art facilities and leadership specific to paediatric rheumatic diseases already are present in the founding groups
- These diseases are dramatically under diagnosed, under treated and poorly understood throughout Asia
- The possibility of networking throughout the continent will also provide the opportunity to achieve the critical mass necessary for translational and clinical research, including clinical trials



CONSENSUS CONFERENCE

Was held on Feb 13th, 2017 (Singapore, Malaysia, Thailand, Philippines, Japan, Australia, China, New Zealand, Europe, the US) to define SOPs for the design of ad hoc translational projects comprising of diagnosis, intervention and collection of samples and clinical data



STUDIES

Tools will be developed to run the studies network-wide. These tools will comprise, but are not limited to, selecting a vendor for the app and developing it, translating and validating questionnaires, harmonising samples shipment procedures



INFRASTRUCTURE

Completing the immunomics infrastructure



BUSINESS DEVELOPMENT

Initiate business development activities to attract funding from local governments, philanthropy and industry



NETWORK

Initiate the procedures necessary to start network activities in the rheumatology and RF areas



TESTING

Test and Validate all tools, SOP and technology platforms in pilot studies in Indonesia and/or other underserved areas



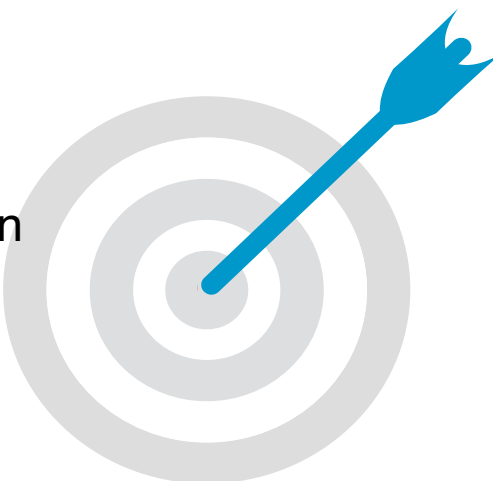
DELIVERABLES OF PHASE 1

SHORT-TERM GOALS:

- A cogent, powerful platform for standardized clinical care and effective translational research.
- A Health Outcome Research platform devoted to Paediatrics.
- A co-ordination between some of the most powerful "Omics" platforms Network-Wide.
- A harmonised processing and analysis of chronic childhood disease samples.
- Run ad hoc pilot studies.
- Train clinicians at various levels for hands-on cutting-edge translational research.
- Pilot project will be in Yogyakarta, Indonesia with 2 local hubs to be built, with potential of Kuala Lumpur, Sudan and UAE being the next
- Recruitment of 100 patients focusing on Paediatric Rheumatology and Rheumatic Fever.

LONG-TERM GOALS:

- Foster a culture of cross-institution collaboration in research on chronic childhood illness.
- Establish a biomedical research Network of Excellence on chronic childhood illnesses.
- Reach out to neighbouring countries to establish trans-national collaboration in important human diseases.
- Global Health Initiative - Be a core contributor towards global health, working with networks spread across Asia, and affiliates in Europe and America.





PHASE 2

- **EXPANSION NETWORK-WIDE**

We will extend the number and scope of the projects network-wide. When possible, we will branch out to underserved areas where local physicians will be provided with appropriate SOPs and be flanked by a nurse who will be assisting in the collection of clinical data and samples and also in the implementation of standard of care.

- **PHILANTROPIC DONATIONS**

This phase will be funded at the national level and we will be actively seeking large philanthropic donations.

- **SELF-SUSTAINED NETWORK**

Of note, at this stage the network should already be self sustaining insofar we could attract significant funding not only from philanthropy and governments, but also industry. The critical mass that we will be able to provide is unequalled worldwide considering the combination of sheer patient numbers of the various countries involved.



PHASE 3

- **IMMUNE-MEDIATED DISEASES**

We aim at expanding the scope of this approach from Paediatric Rheumatology and Rheumatic fever to a broader array of immune-mediated diseases including but not limiting to cancer, allergy and infectious diseases.



OVERALL DELIVERABLES

- **World-class, Asia-wide network**

State-of-the-art care and translational research will be conceived and executed. In this context, the Network will be transformational for the delivery of Health Care and development of Research.

- **Opportunity 1**

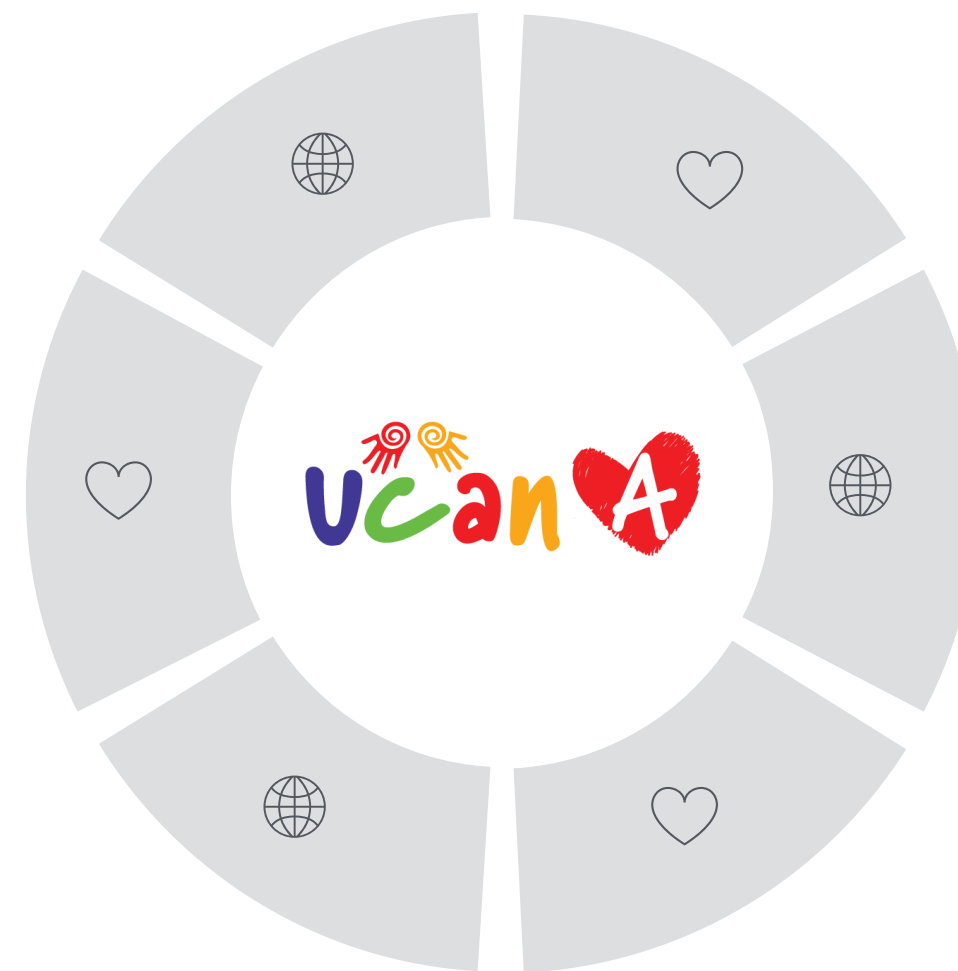
To provide standard of care to underserved populations and, in general, to improve health outcomes in Paediatrics.

- **Opportunity 2**

To provide a platform for Health Care Outcomes Research in Paediatrics.

- **Opportunity 3**

To provide an unrivalled, hypothesis and medical needs inspired “omics” platform in Pediatrics



- **Opportunity 4**

To offer to Industry the largest paediatric population of patients for clinical trials in a well standardized setting.

- **Opportunity 5**

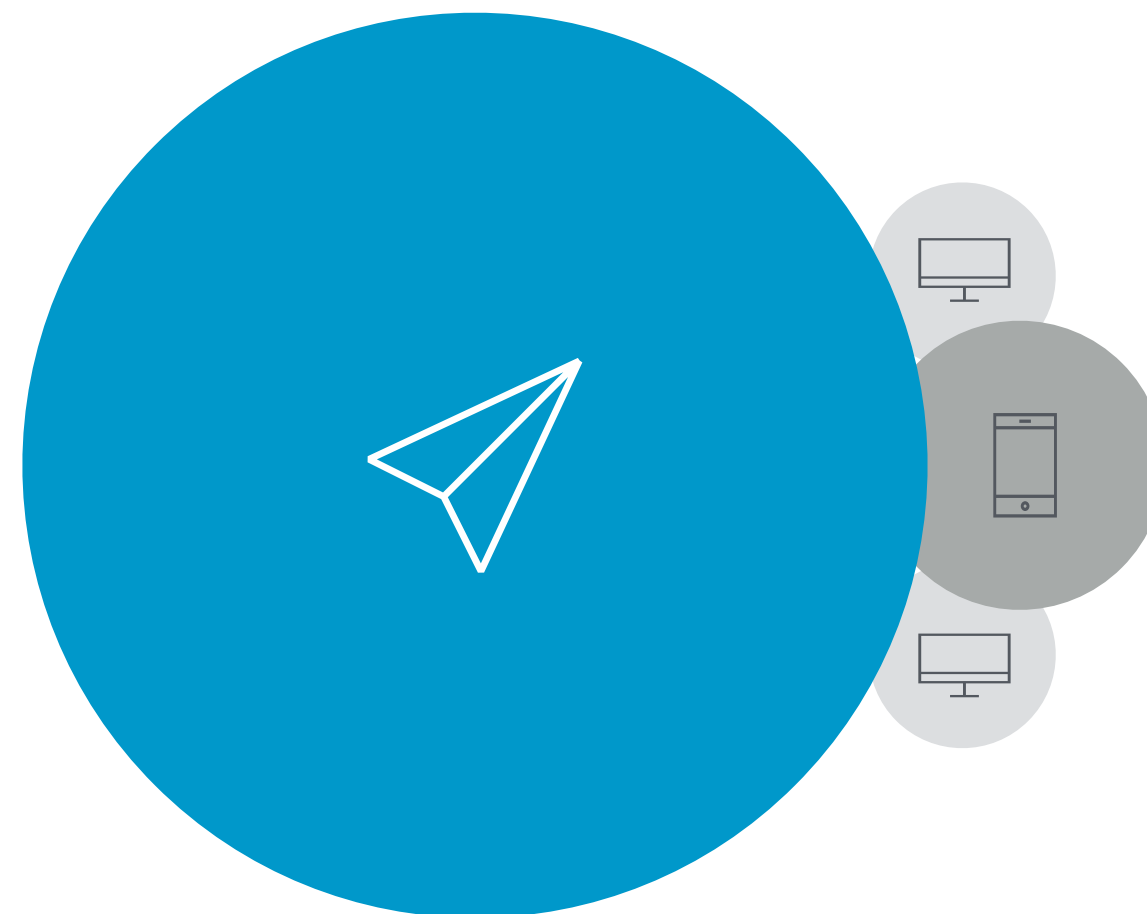
To provide opportunities for medical education at various ranges of expertise.

- **Opportunity 6**

A unique platform for transformational Translational Research, leading to addressing unmet needs by identifying new biomarkers and therapeutic targets, thus bringing tangible benefits both financially and for the patients.



UNDERSTANDING CHILDHOOD (CHRONIC) AILMENTS NETWORK - ASIA (UCAN-A)



ALTOGETHER, UCAN-A WILL BE:

Impactful,

as it will affect many lives in diverse and finally integrated fields, ranging but not limited to:

- provision of care to the underserved;
- opportunities for education of patients and their families;
- opportunities for education of medical professional at any level of training;
- opportunities to perform meaningful translational research;
- opportunities to perform large clinical trials while covering also analysis of mechanism and biomarkers.

Self Sustainable,

as it will attract Industry funding, due to its unrivalled critical mass, and philanthropy, due to its unrivalled reach to underserved population.

Transformational,

as it will truly change many lives, ranging from patients and families to care providers to scientists. UCAN-A will impact the way diagnoses will be made and therapies administered, contributing to the evolution from phenotype-based decisions to knowledge, funded practice.



UNMET MEDICAL NEEDS

Patients

Technologies

Does Technology reduce or broaden
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UNMET NEEDS

DUAL TRANSLATIONAL GOALS:

IDENTIFICATION OF THERAPEUTIC TARGETS

PREDICTION OF CLINICAL FATE

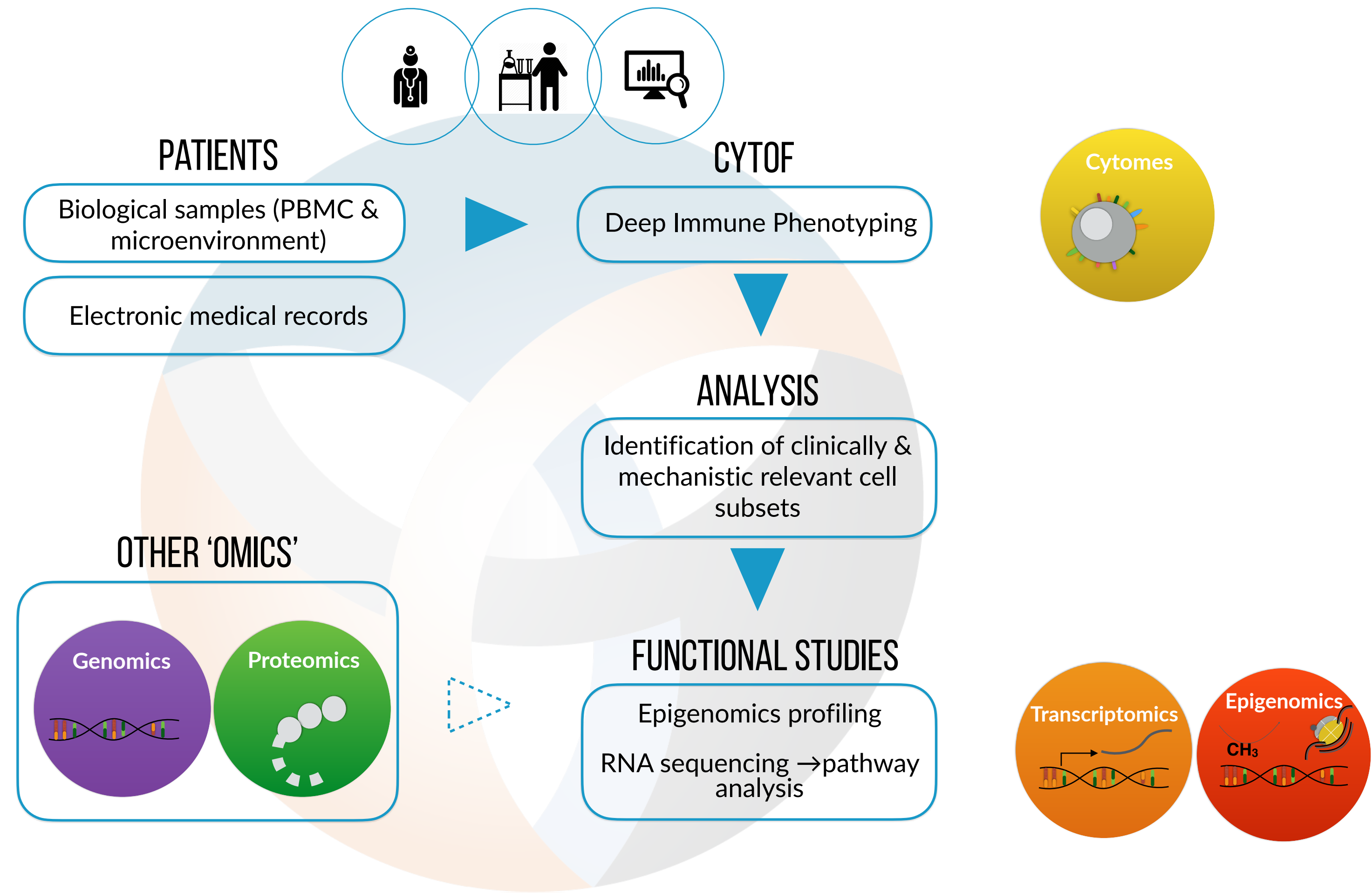
LOOKING AT THE HUMAN IMMUNOME

WE HAVE DEVELOPED AN ATLAS OF THE HEALTHY HUMAN
IMMUNOME

WE CAN USE THIS ATLAS TO PREDICT CLINICAL FATE

WE CAN USE THIS ATLAS TO LOOK AT THE EFFECTS
OF “PERTURBATIONS”, SUCH AS DISEASE OR THE
EFFECTS OF IMMUNE THERAPY

IMMUNOMICS PLATFORM





DISEASES AS PERTURBATIONS OF THE HUMAN IMMUNOME: PREDICTORS OF CLINICAL FATE IN HUMAN ARTHRITIS

UNMET NEEDS:

A GOOD PROPORTION OF ARTHRITIS PATIENTS RESPOND TO ANTI-TNFA, BUT 50-80% FLARE UPON DRUG DISCONTINUATION. THERE ARE NO CLEAR PREDICTORS TO INFORM CLINICAL MANAGEMENT.

HYPOTHESIS:

CD4 T CELLS ARE LIKELY ONE OF THE MAJOR MECHANISTIC DRIVERS FOR DISEASE RESURGENCE AND COULD POTENTIALLY SERVE AS DISCRIMINATORY TOOL TO DETERMINE CLINICAL FATE

APPROACH:

TRIAL: POLYARTICULAR JIA IN REMISSION WITH ANTI-TNFA ARE WITHDRAWN AND SAMPLED PRIOR AND AFTER WITHDRAWAL OF THERAPY. IMMUNOMICS: TO OBSERVE HOW THE CD4 LANDSCAPE IS RESHAPED UPON INTERVENTION



CONCLUSIONS

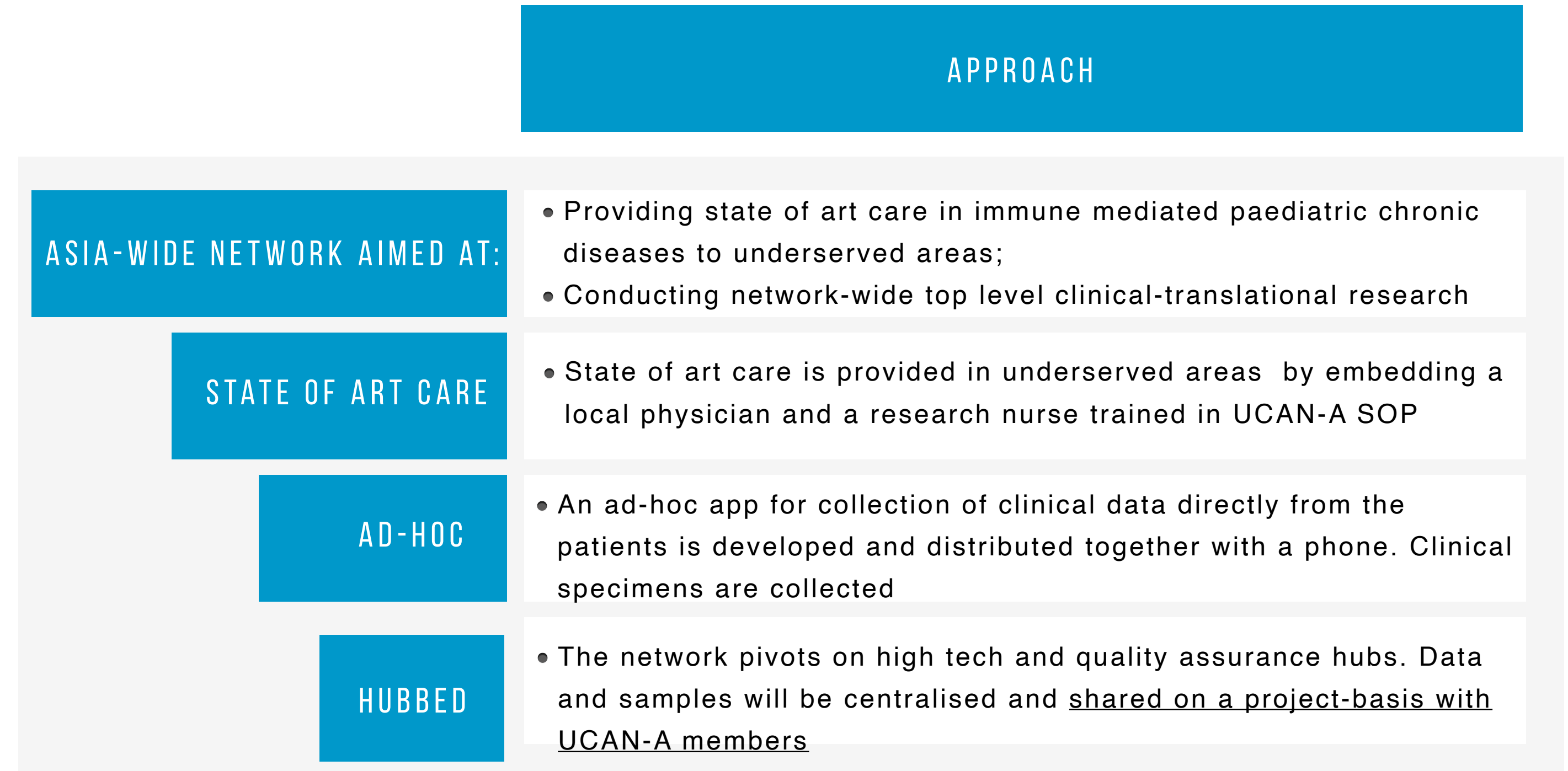
INTERSECTION AND INTEGRATION OF BIG DATASETS LEADS TO THE HOLISTIC VIEW OF UNDERLYING DISEASE MECHANISMS CAN SIGNIFICANTLY SPEED UP DELIVERY OF STAT OF ART CARE IN THE CONTEXT OF UCAN-A.

GOALS:

- DISEASE STRATIFICATION (MOLECULAR TAXONOMY THAT AFFECTS THERAPEUTIC CHOICES)
- PREDICTION OF CLINICAL FATE
- IDENTIFICATION OF NOVEL THERAPEUTIC TARGETS
- INTEGRATION OF THE OMICS DATA WITH HEALTH DELIVERY OUTCOMES COLLECTED FROM THE SAME POPULATION IN A HIGHLY CONTROLLED NETWORK



UCAN-A APPROACH



PATIENTS. AT THE HEART OF ALL WE DO.®



THANK YOU