Implementation of CAR-T Cell Therapy in Costa Rica

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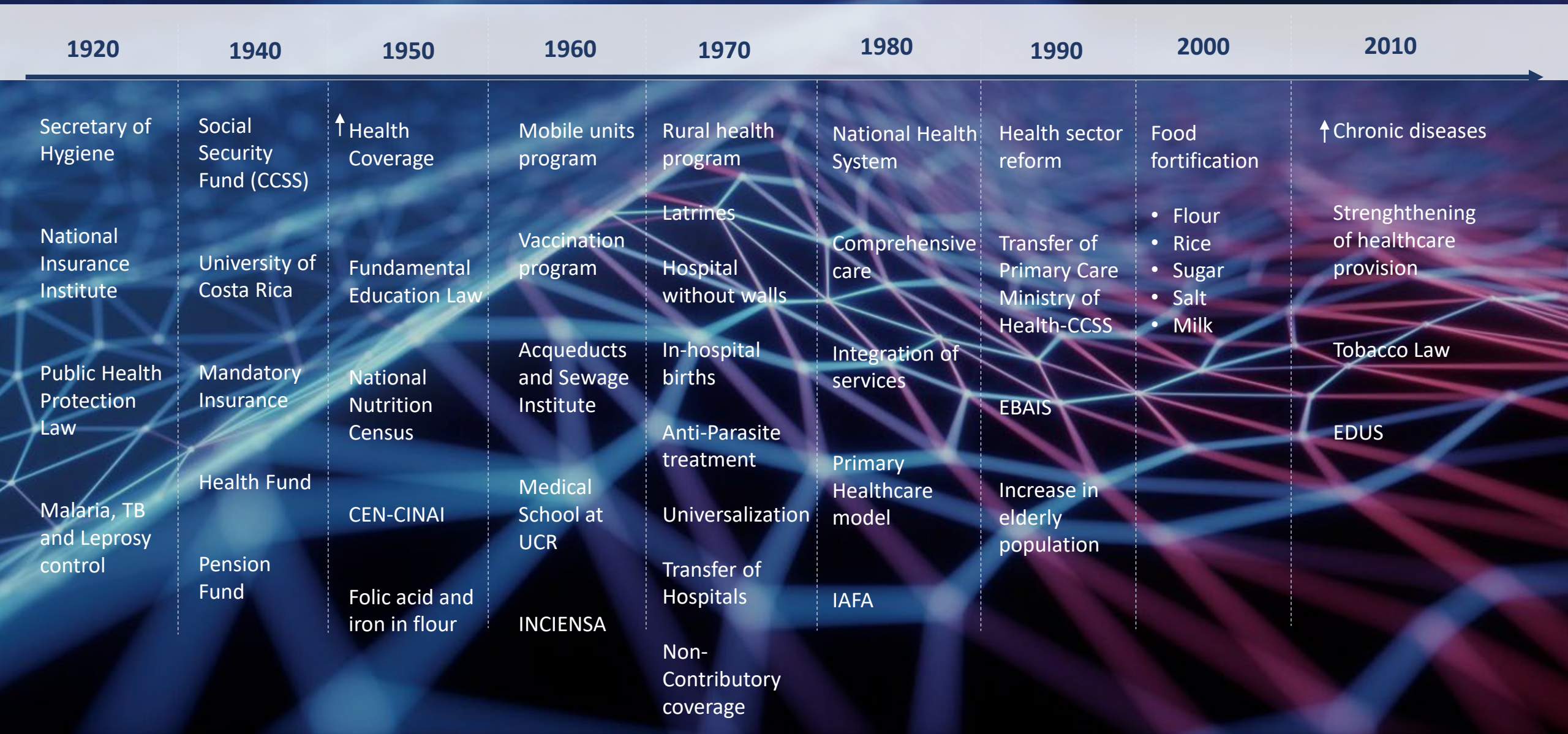
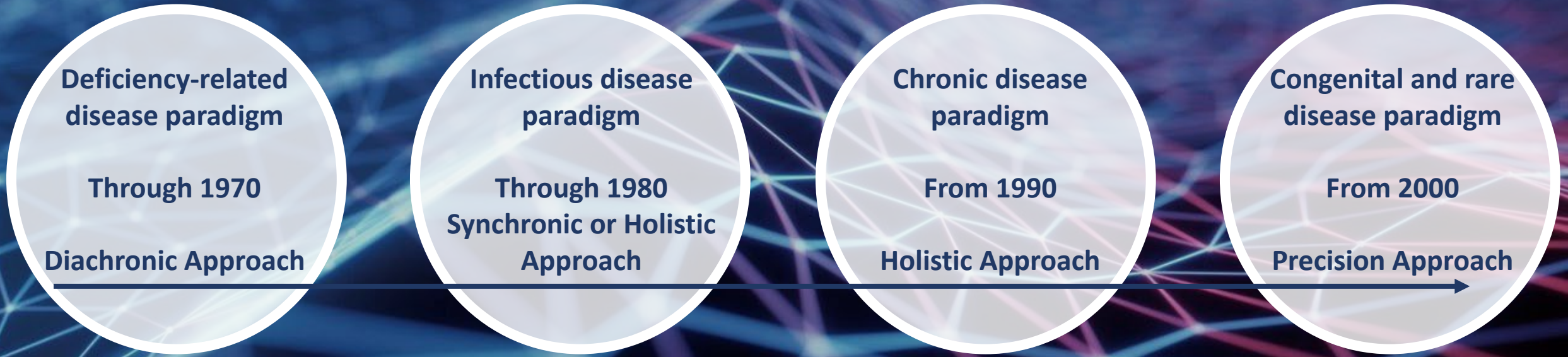
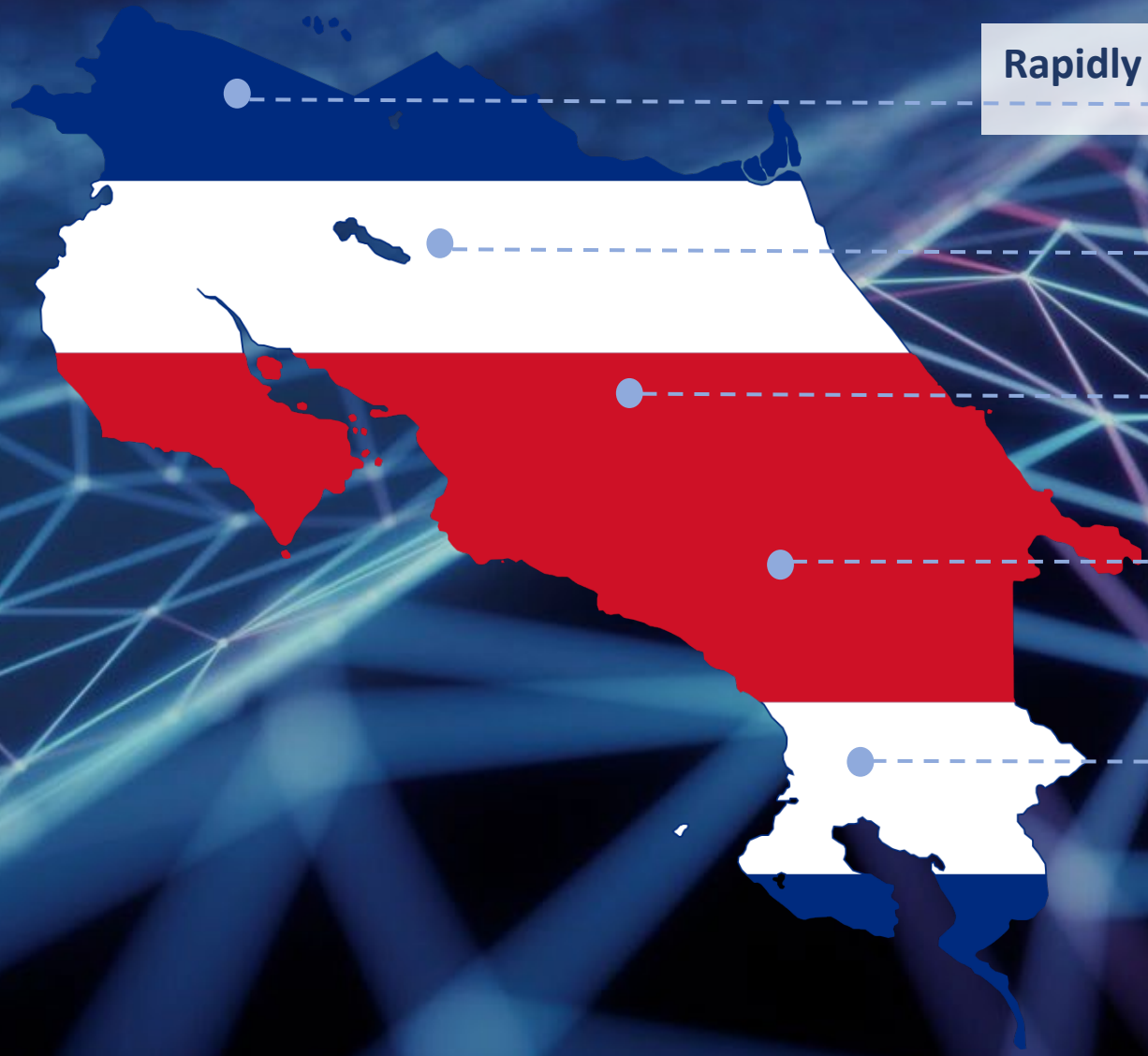
Costa Rica´s notable health indicators are a result of historical and political commitments



Costa Rica has seen rapid changes in health paradigms towards rare and congenital diseases



Costa Rica´s health system faces serious challenges for sustainability and access to health services



- Rapidly aging population
- Burden of chronic disease
- Constitutional mandate for health (Article 21)
- Judicialization of health
- Cost of new technologies: cell and gene therapies

Costa Rica has 1-2 possible pediatric candidates for CAR-T cell therapy per year

Year	Leukemia Cases	B-Cell ALL # (%)	T-Cell ALL # (%)	Other	Refractory
2016	66	51 (77.2)	2 (3)	13 (19.7)	2
2017	63	51 (81)	4 (6.3)	8 (12.7)	2
2018	60	50 (83.3)	2 (3.3)	8 (13.4)	1
2019	69	53 (76.8)	4 (5.8)	12 (17.4)	2

Collaboration with the University of Pennsylvania and the Children's Hospital of Philadelphia is strategic for CAR-T implementation in Costa Rica

- Initial visits to UPenn and Dr. June´s Lab by Costa Rican Embassy in the U.S. (2015, 2016, 2017)
- Visits to Costa Rica's National Children's Hospital by delegations from UPenn and CHOP, including Dr. Carl June, Dr. Bruce Levine, Dr. Glen Gaulton and Dr. Stephan Grupp (2016, 2017, 2018)
- Memorandum of Understanding between UPenn, CHOP and CCSS (Pending)
- Wharton students´ Field Application Project, members of Dr. June´s Lab and Dr. Stephan Grupp (CHOP) visit Costa Rica in February 2020

Before COVID-19, implementation of CAR-T was planned in three stages

Initial Stage
The Index Case

- CHOP-UPenn-CCSS Memorandum of Understanding
- Index case(s) (1-2)
- Training visits for index cases

Mid-Stage
Implementation and local accreditation

- Accreditation of apheresis
- Certification of post infusion care
- Cell transformation abroad

Advanced Stage
Consolidation and growth

- National Apheresis and Cellular Therapy Center
- Local apheresis, transformation, infusion and hospital care
- Adult pathologies
- National and international research

The COVID-19 pandemic has meant a hard reset on the timeline

Leukemia patients require access to more therapeutic options and a higher standard of care

Equity and Equality are two of the seven principles of the "Caja Costarricense de Seguro Social"

Objectives

- Improvements in infrastructure; better isolation conditions
- Improved access to drugs
  - Busulfan IV - Midostaurin
  - Mitoxantrone - Procarbazine
  - Thiotepa - Monoclonal antibodies
- Improvements in Hematopoietic Stem Cell Transplantation (HSCT) programs
- Planned access to therapies not offered today
  - CAR-T cell therapy
  - Therapeutic photo-apheresis
  - Total Body Irradiation

Challenges

- FACT-JACIE accreditation for apheresis

  (National accreditation plan for apheresis?)
- Cell transformation facility (GMP accreditation?)

The National Children´s Hospital of the CCSS aims to offer the highest standard of care in pediatrics, including precision medicine, research and innovation

Future vision considers cell and gene therapy, a genomics center, and biomedical research

National Neonatal Screening Program founded in 1990 currently screens for 51 congenital diseases

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Published/Presented Results in ALL

Sponsor	NCT	Age	Antibody	Co-stim	Integration	CR rate	Durability
SCH n=43	02028455	1-26 y	FMC63	4-1BB	Lenti	93%	1 year EFS 50.8%
FHCRC n=29	01865617	≥ 18 y	FMC63	4-1BB	Lenti	94%	1 y DFS ~65% (flu/cy)
CHOP n=59	01626495	1-24 y	FMC63	4-1BB	Lenti	93%	1 year RFS 55%
UPENN n=27	01029366 02303847	21-72 y	FMC63	4-1BB	Lenti	56%	NR
Novartis n=75	02435849	3-21 y	FMC63	4-1BB	Lenti	81%	1 year EFS 50%
MSKCC n= 46	01044069	≥ 18 y	SJ251	CD28	Retro	83%	6mo OS 57% (>5%dz)
NCI n=51	01593696	Peds/YA	FMC63	CD28	Retro	60%	Median OS 13.3mo
CHOP n=22	02374333	Peds/YA	humanized	4-1BB	Lenti	100%	RFS at 12 months 82%

SCRI Seattle Children's Research Institute (Gardner et al Blood 2017); FHCRC Fred Hutchinson Cancer Research Center (Turtle et al JCI 2016); CHOP Children's Hospital of Philadelphia (Maude et al ASCO 2016) ; UPENN University of Pennsylvania (Frey et al ASCO 2016); Novartis (Maude et al NEJM 2018) MSKCC Memorial Sloan Kettering Cancer Center (Park et al ASCO 2016); NCI National Cancer Institute (Lee et al. ASH 2016)