Improving Research in Pediatric Nephrology

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NATIONWIDE CHILDREN'S
When your child needs a hospital, everything matters.℠

THE OHIO STATE UNIVERSITY
COLLEGE OF MEDICINE

Objectives

ROADMAP

1. Identify six megatrends in child health for the near future
2. Define six top targets for Pediatric Nephrology research in the near future
3. Describe key research approaches in Pediatric Nephrology and consider, how, by joining in you can improve research in pediatric nephrology in the future!

History's path is unchartable
when it is not yet past, but present,
when we are standing in the middle of it.

Jennifer Couzin-Frankel
Science 342: 1432, 2013

Regarding cancer immunotherapy
the 2013 *Science* Breakthrough of the Year

Shout out to **John Barnard, MD** (Chair Department of Pediatrics at The Ohio State University College of Medicine) and **John David Spencer MD** (Division Director, Pediatric Nephrology, OSU)

Six Megatrends in Pediatric Health Care

Population health and health equity

Genomics

Treatment of rare diseases by gene therapy

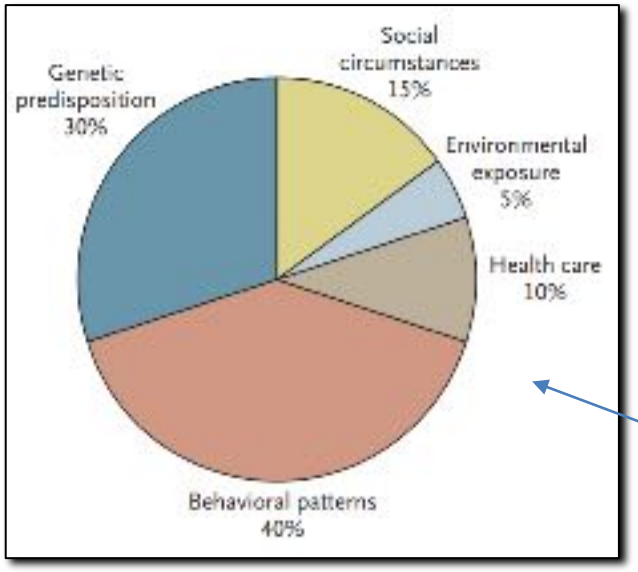
Behavioral and mental health

New diseases, undesirable trends and unplanned events

The pediatric subspecialty workforce

Population Health and Health Equity

"We Can Do Better"



Determinants of Health and
Their Contribution to Premature Death

We Can Do Better — Improving the Health of the American People
Steven A. Schroeder, M.D.

Structural and individual racism

N Engl J Med 2007; 357:1221-1228

Population Health

"Inequities in power, money and resources give rise to inequities in the conditions of daily life, which in turn lead to inequities in health"

Sir Michael Marmot
Professor Epidemiology and Public Health
University College of London

"If zip code is an enduring driver of both short-term and long-term health, children's hospitals and other health care facilities as anchor institutions should be in the zip code improvement business."

Kelly Kelleher, MD MPH
The Healthy Neighborhood, Healthy Families Initiative
Pediatrics 2018, 142 (3) e20180261

"Moral Determinants of Health"

Donald Berwick, MD MPP
President Emeritus and Senior Fellow
Institute for Health Care Improvement

Definition: Population Health

Population health is a commitment to addressing the social (and moral) determinants of health and preventing illness before it develops.

Examples of social determinants that may be targeted by hospitals in population health approaches include housing, transportation, education, employment, and access to food and exercise.

Genomics

20 Year

Anniversary

Genomics is the study of all of a person's genes (the genome), including interactions of those genes with each other and with the person's environment.

Harnessing the full potential of genomics to improve health is an endeavor that will span decades, if not centuries.

Feero and Gutmacher
Acad Pediatr 2014 14:14-22.

Genomics


Preconception and Prenatal Testing
Newborn Screening
Disease Susceptibility
Screening and Diagnosis
Prognosis and Therapeutic Decisions
Monitoring Disease Burden and Recurrence
Genomics

Treatment of Rare Diseases by Gene Therapy

Rare diseases are not rare, at least in aggregate. Approximately 7,000 rare diseases afflict millions of individuals in the United States and are responsible for untold losses in terms of physical health, behavioral health, and socioeconomic condition.

Thomas F. Boat, MD Chair Committee on Accelerating Rare Diseases Research and Orphan Product Development

Rare and neglected diseases are largely the singular domain, concern and responsibility of pediatricians. They are in no small part what define us among all physicians.

John Barnard, MD

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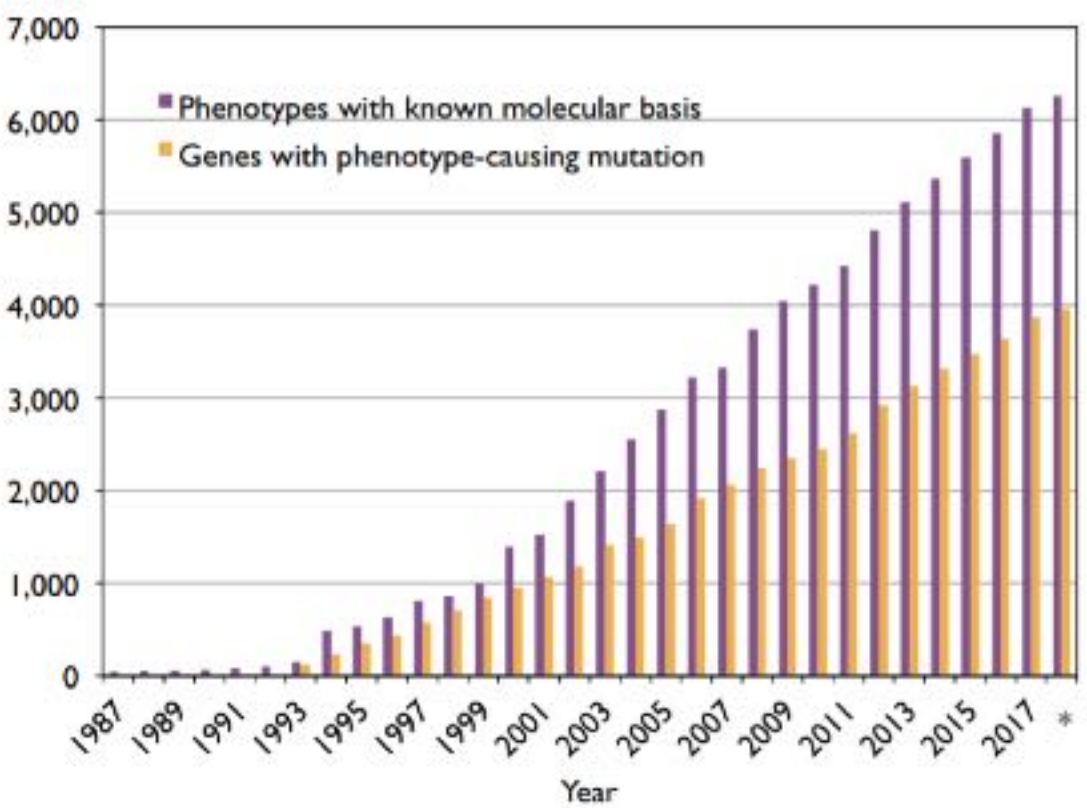
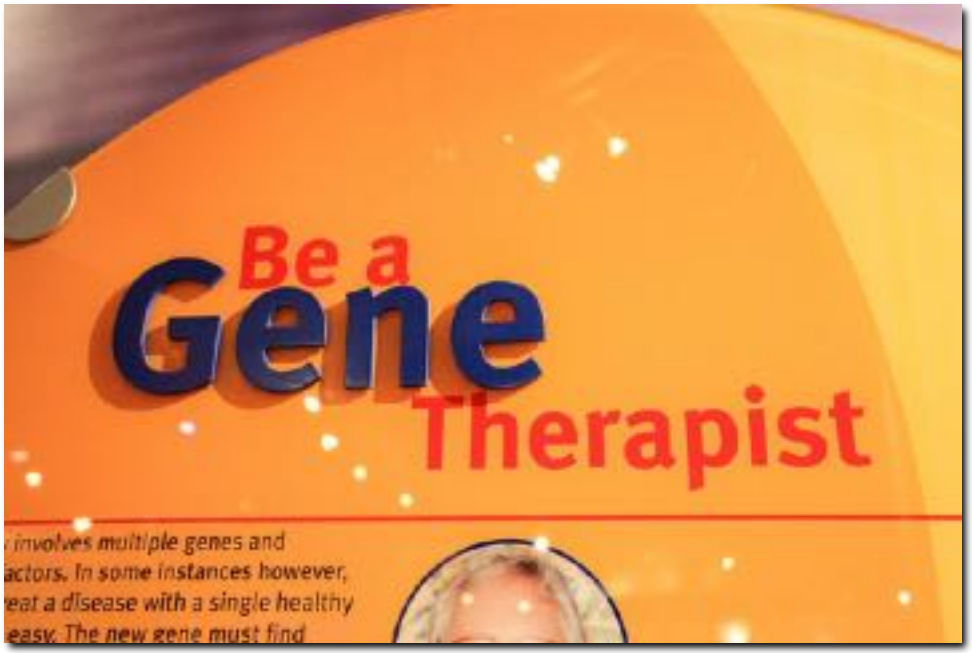
Growth of Gene-Phenotype Relationships



Nucleic Acids Research, 2019, Vol. 47, Database issue

Source: Online *Mendelian Inheritance in Man*

Gene Replacement Therapy



FDA-Approved
Gene Replacement Therapies

Luxturna (2017)
voretigene neparvovec ($425K/eye)
Children's Hospital of Philadelphia
Leber congenital amaurosis
AAV

Zolgensma (2019)
onasemnogene abeparvovec ($2.1M)
Nationwide Children's Hospital
Spinal muscular atrophy
AAV

Gene Replacement Therapy: Future

Worldwide Implications

Multiple “blockbuster” therapies will reach market in next decade

Huge commercial interests; how these are disseminated throughout the world

Optimization of methods: viral and other vectors

Improved manufacturing efficiency

Long term efficacy

Price considerations

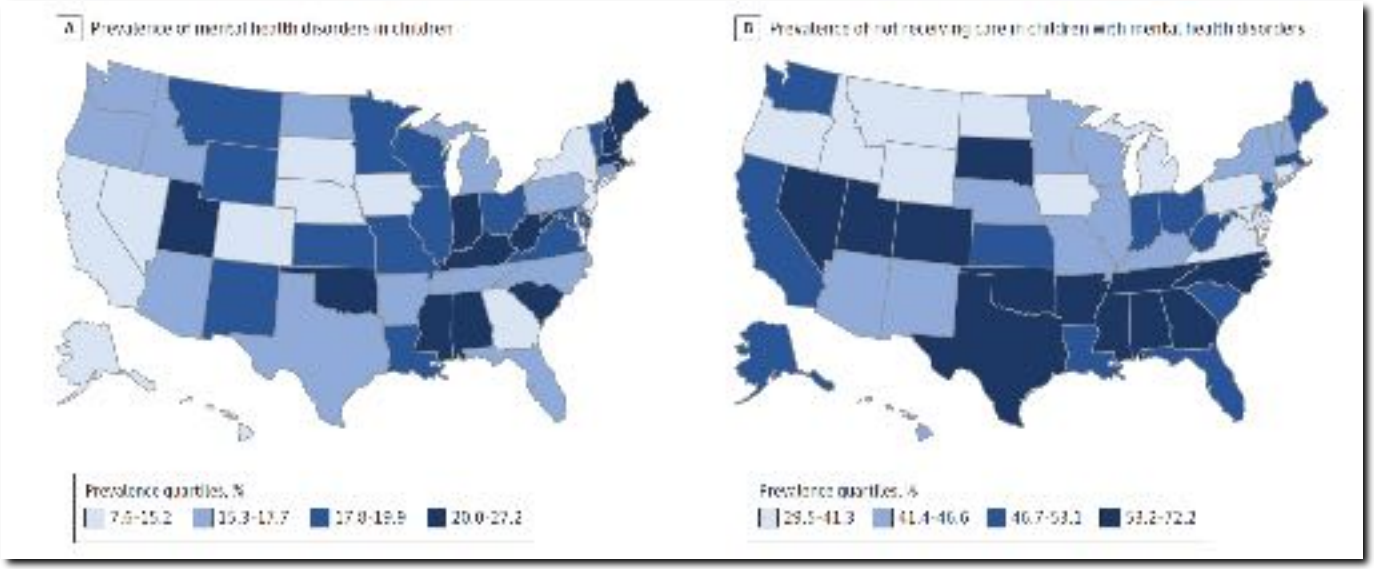
Behavioral and Mental Health

The full effect of COVID-19 on children's behavioral health likely won't be known for some time. But even before the pandemic, pediatric behavioral health was a national crisis, with 1 in 5 children [in the US] living with a mental illness and suicide the second leading cause of death for children 10 and older.

Children's Hospital Association

Prevalence of Pediatric Mental Health Disorders

16%



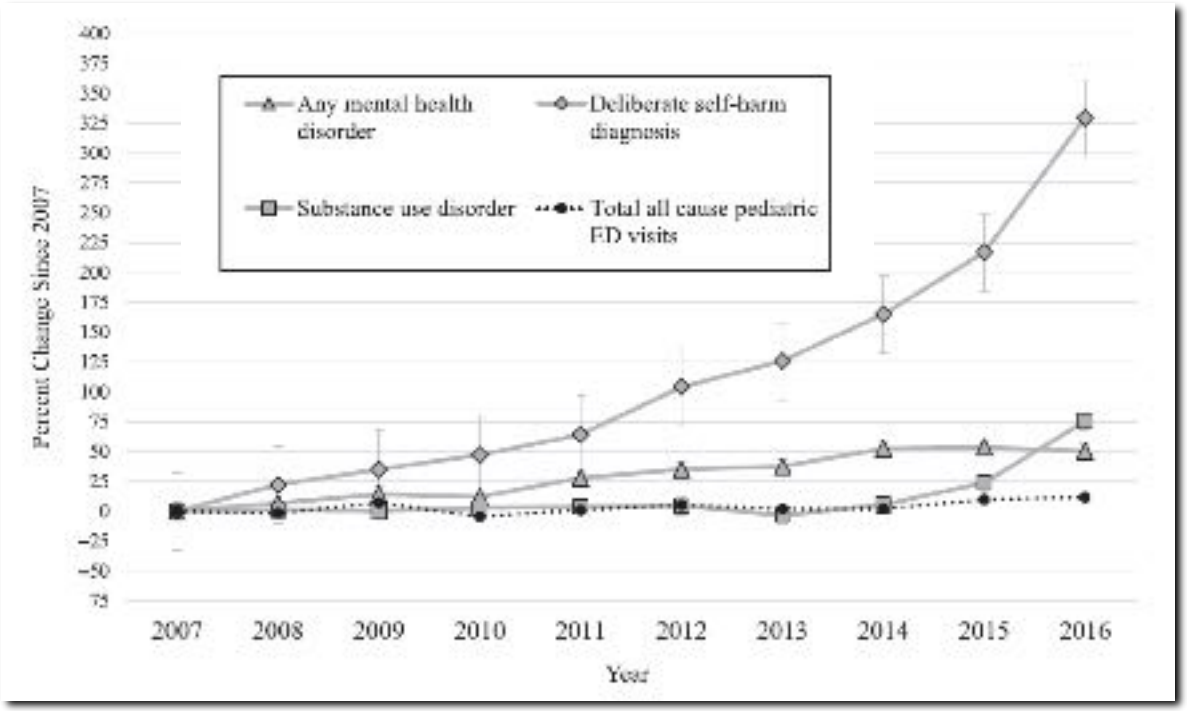
49%

~ 50,000 surveys : National Survey of Children's Health
JAMA Pediatrics April 2019 Volume 173, Number 4

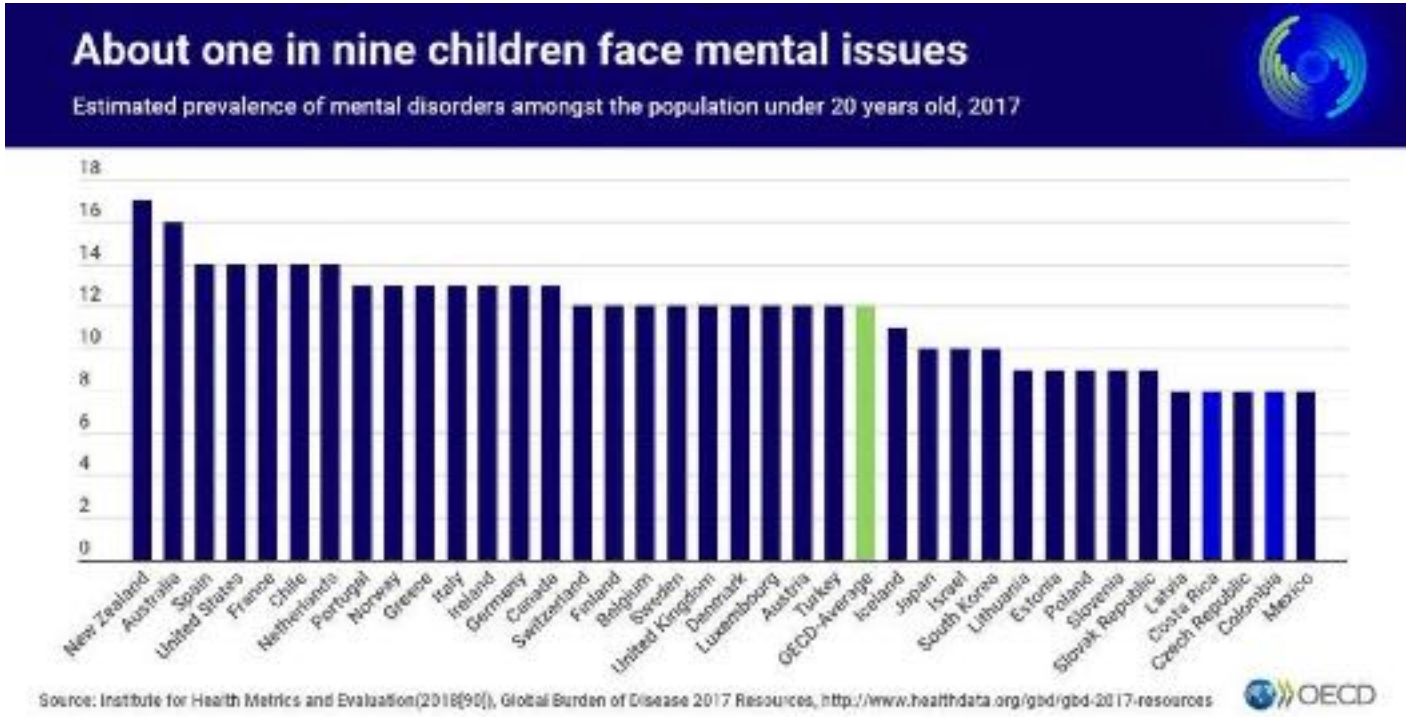
Children's Mental Health Emergency Department Visits: 2007–2016

Charmaine B. Lo, PhD, MPH,[a] Jeffrey A. Bridge, PhD,[b,c] Junxin Shi, MD, PhD,[a,f] Lorah Ludwig, BS,[a] Rachel M. Stanley, MD, MHSA[a,e]

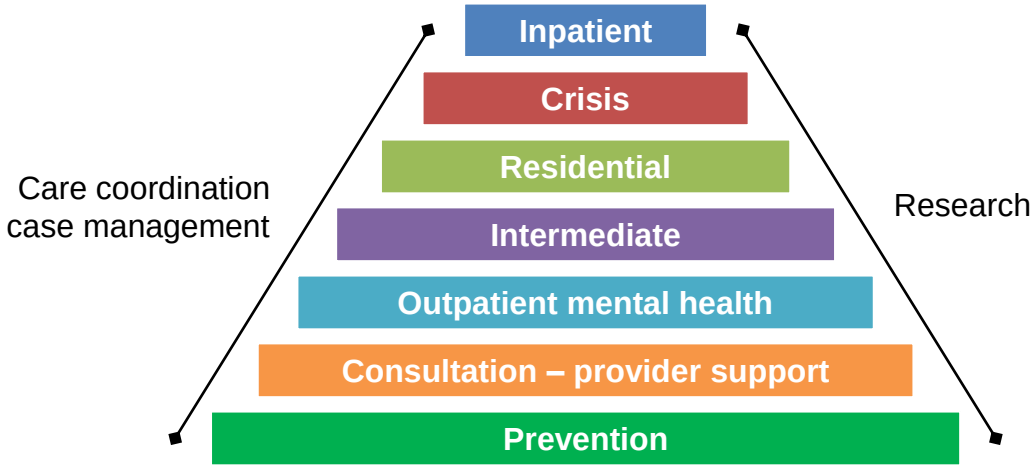
PEDIATRICS Volume 145, number 6, June 2020:e20191536



Mental Health Disorders in Children



Behavioral and Mental Health: Future



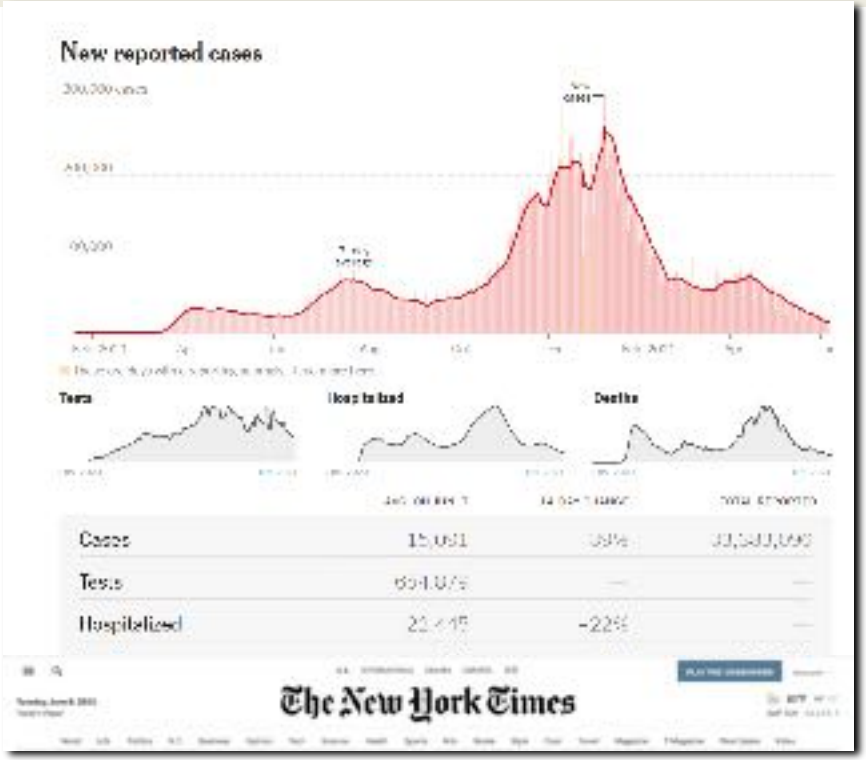
Big Lots Behavioral Health Pavilion

New Diseases, Undesirable Trends, and Unplanned Events

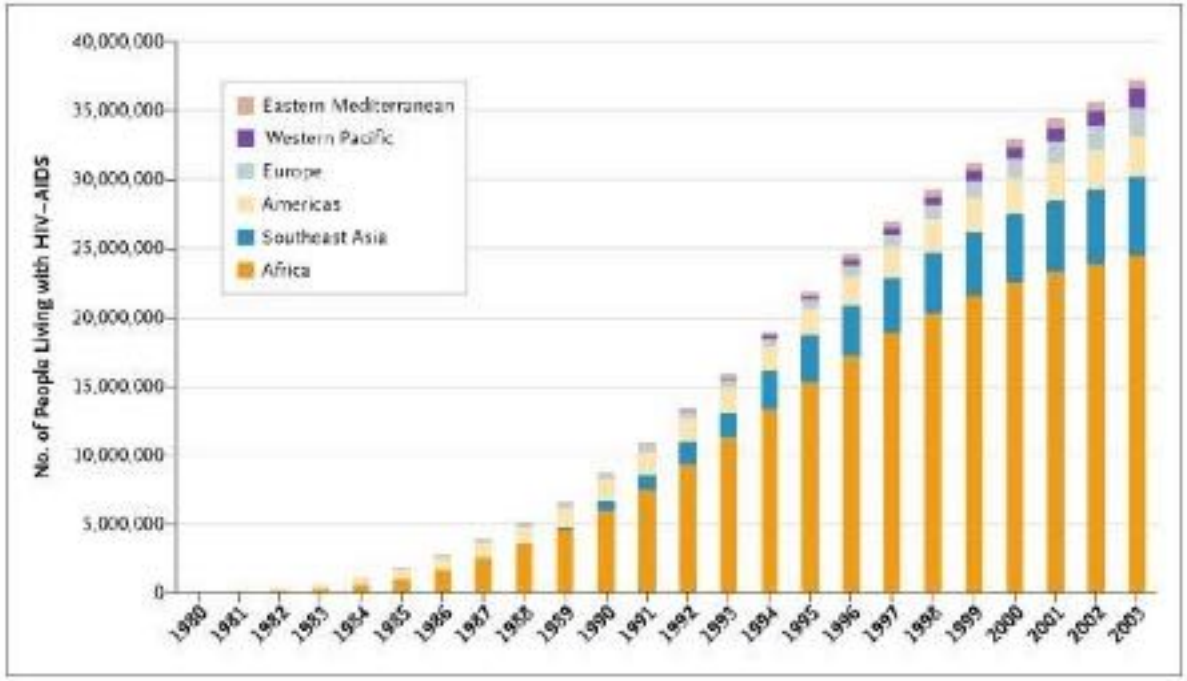
Ch-ch-ch-ch-changes
(Turn and face the strange)
Ch-ch-changes
Oh, look out you rock 'n' rollers

David Bowie, 1971

SARS-CoV-2

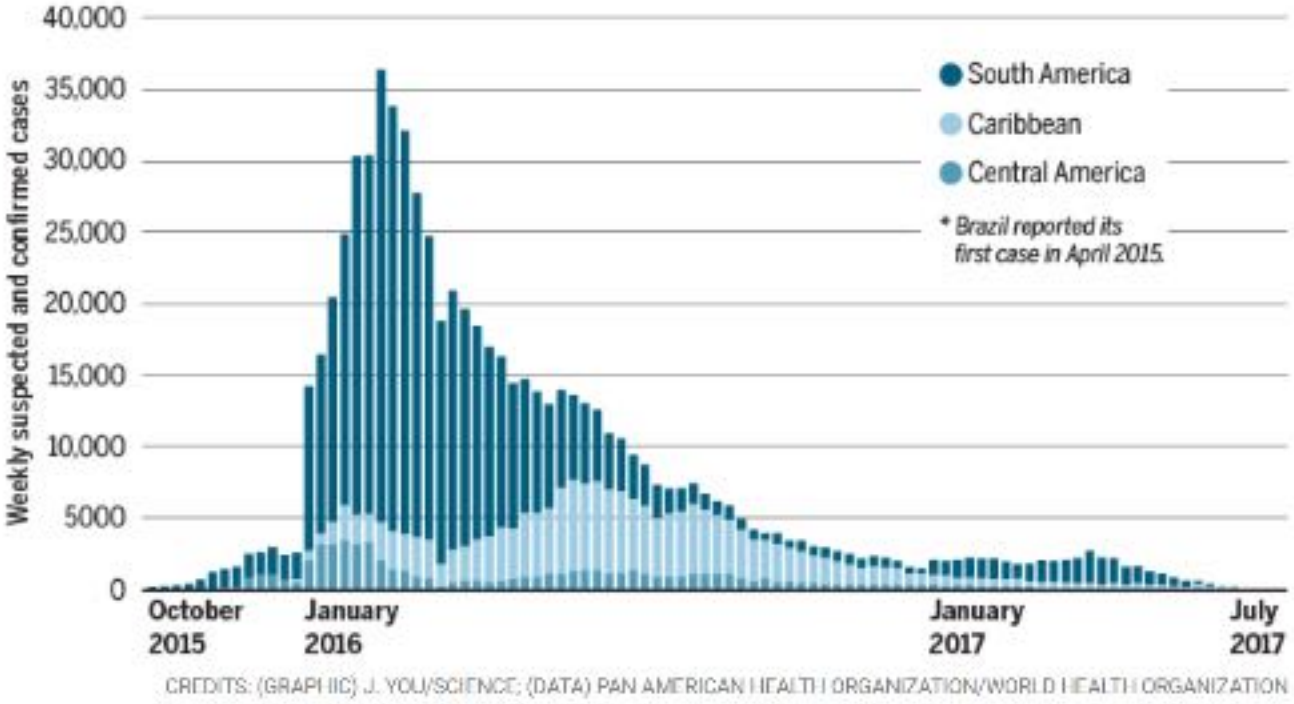

New reported cases
Cases
Tests
Hospitalized
The New York Times

HIV



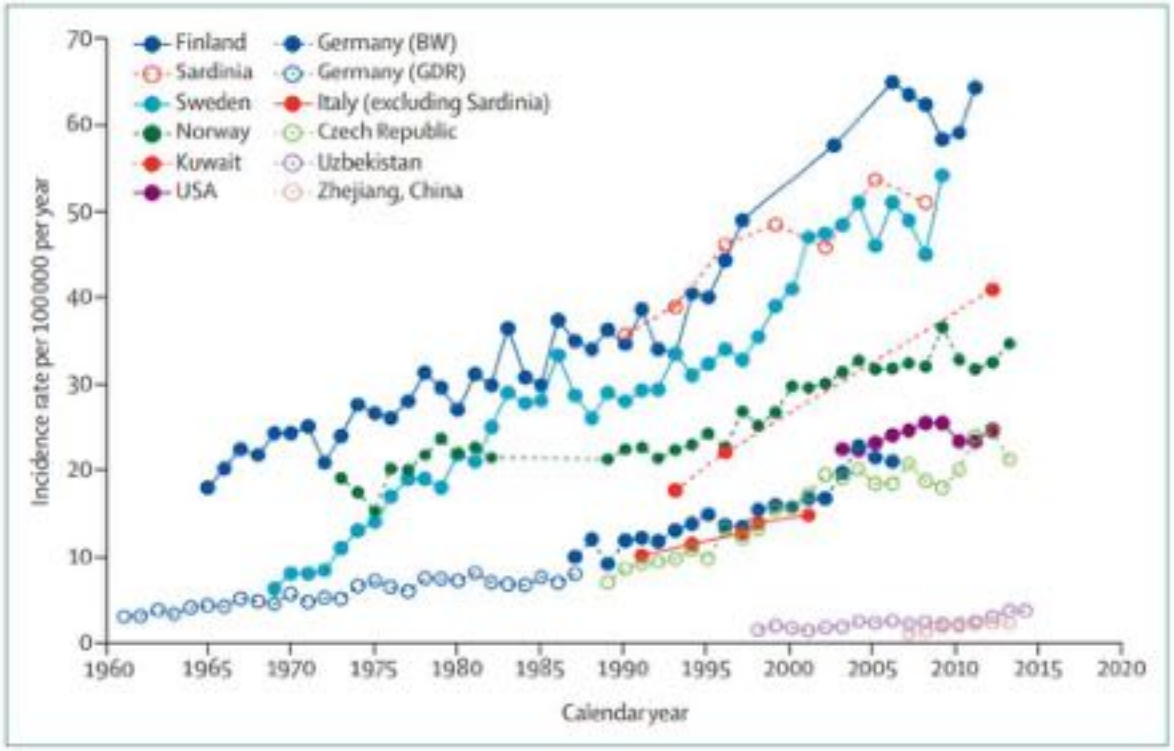
Steinbrook R. *N Engl J Med* 2004;351:115-117.

Zika Virus

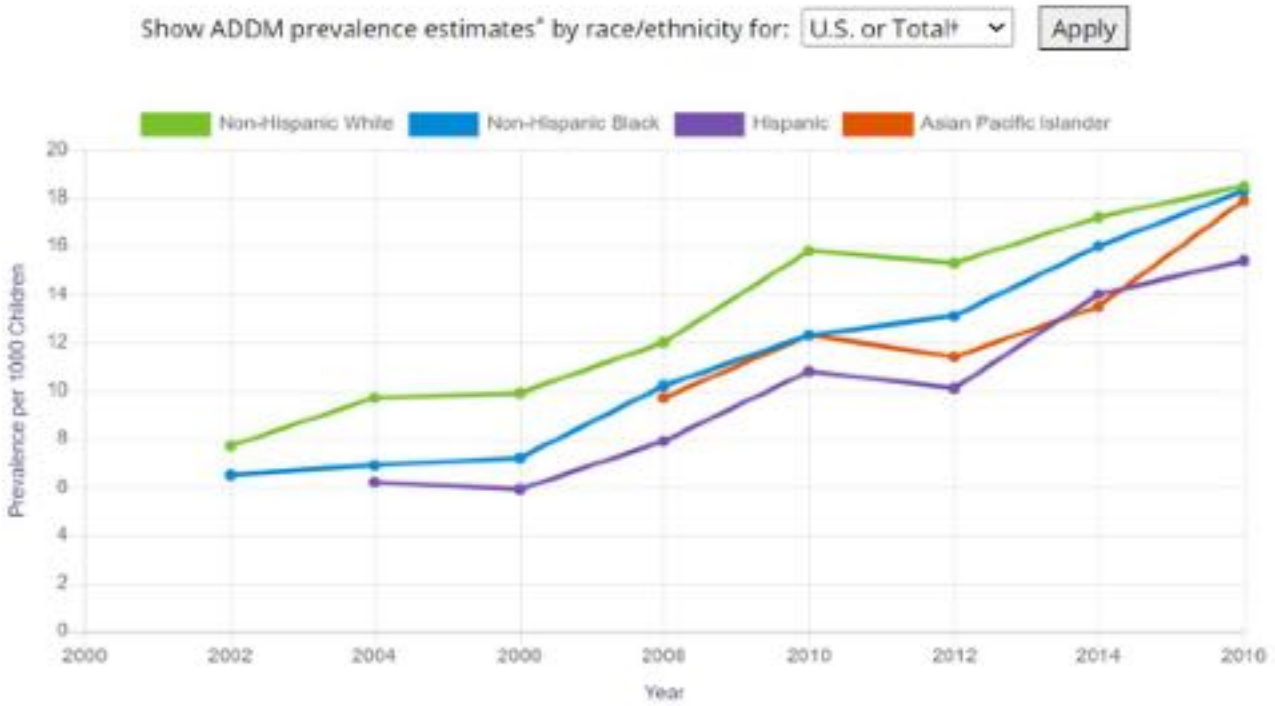


https://www.sciencemag.org/news/2017/08/zika-has-all-disappeared-americas-why

Type 1 Diabetes

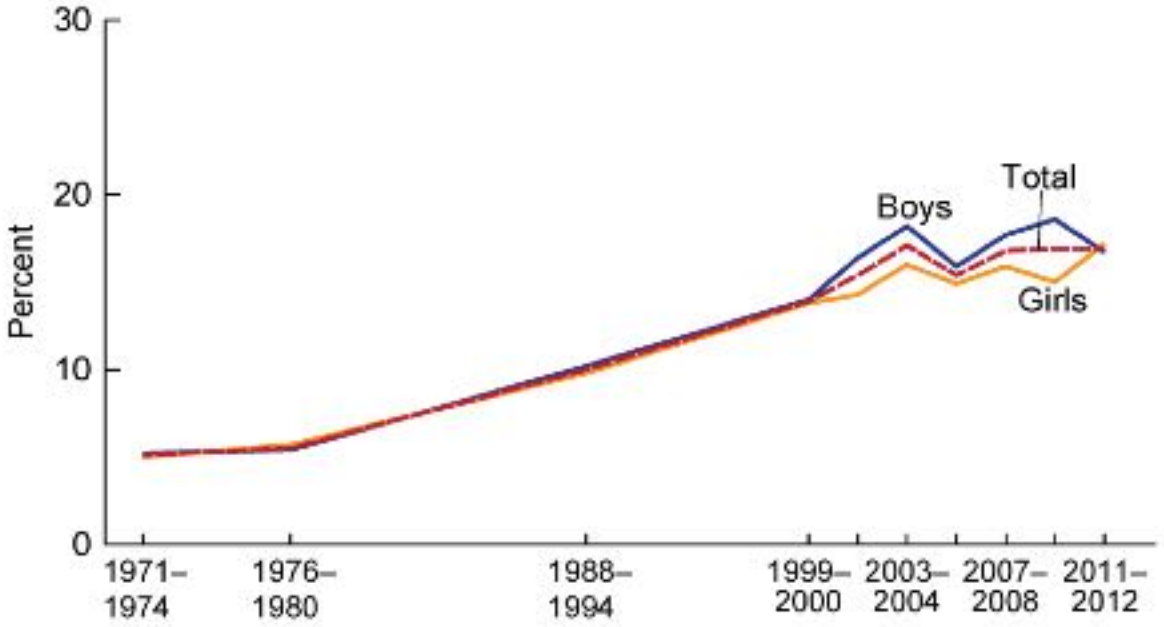


Autism: Prevalence by Race/Ethnicity



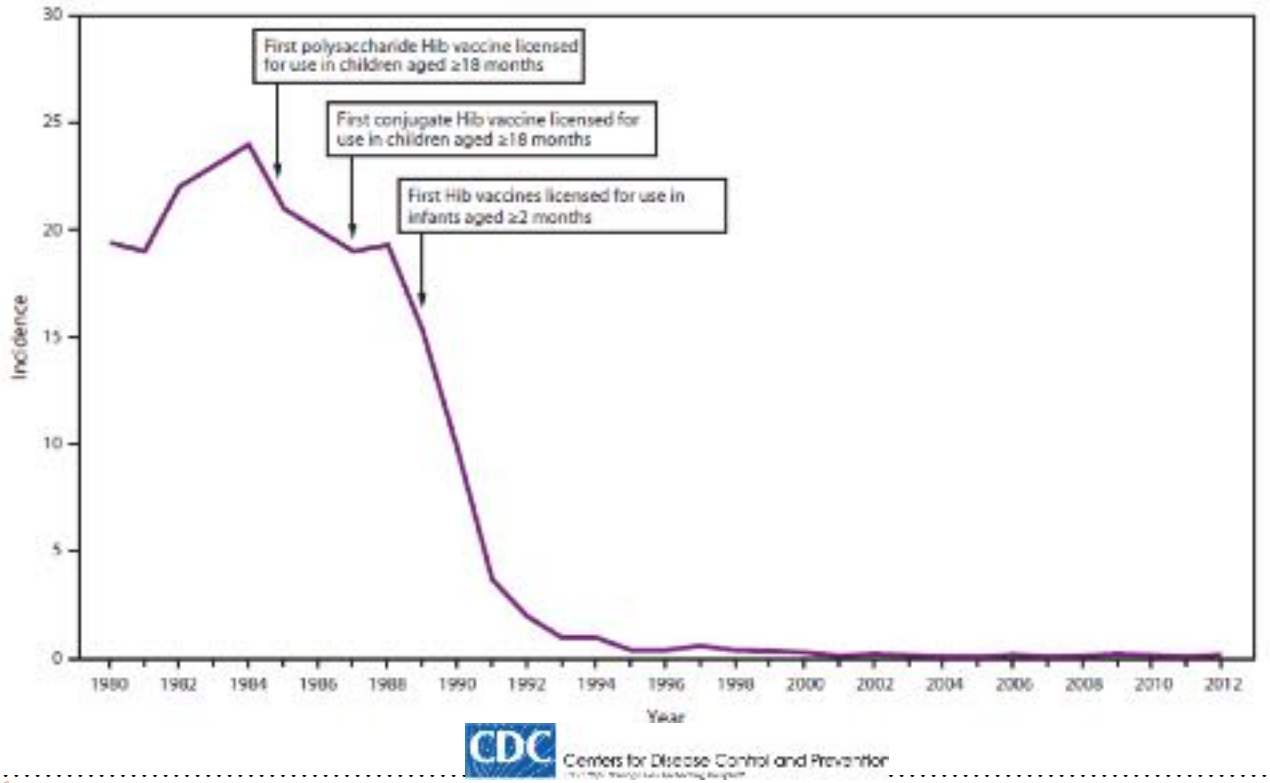
https://www.cdc.gov/ncbddd/autism/data/index.html

Trends in Obesity: Age 2-19



SOURCE: CDC/NCHS, National Health and Nutrition Examination Surveys

Annual Incidence Hib Age < 5 yr



CDC Centers for Disease Control and Prevention

Preparation for Unexpected Events

Achieve financial stability (weather downturns)

Recruit top talent – physician, nursing, admin, other

Invest aggressively in research – anticipate the future

Plan strategically – future oriented

Continuously benchmark

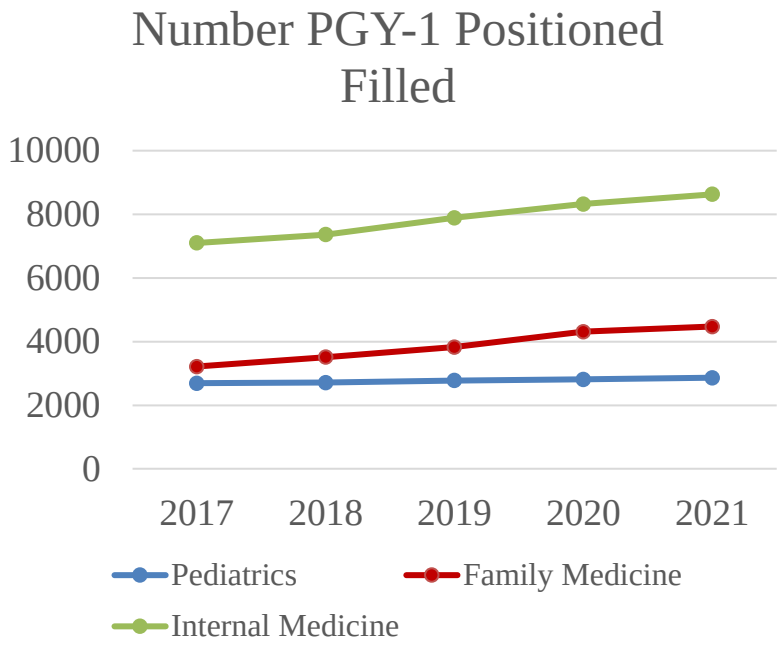
Constantly assess and monitor risks

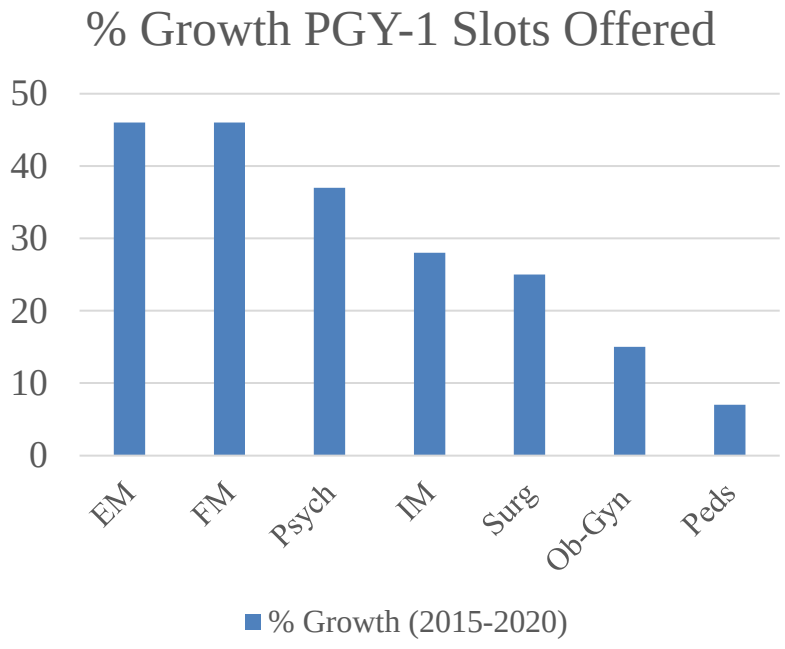
Build organizational capacity

Promote robust, nimble, team-oriented healthcare culture

Having Sufficient Pediatric Subspecialists in the World

Pediatrician Workforce: Trends in Residency Training in the US

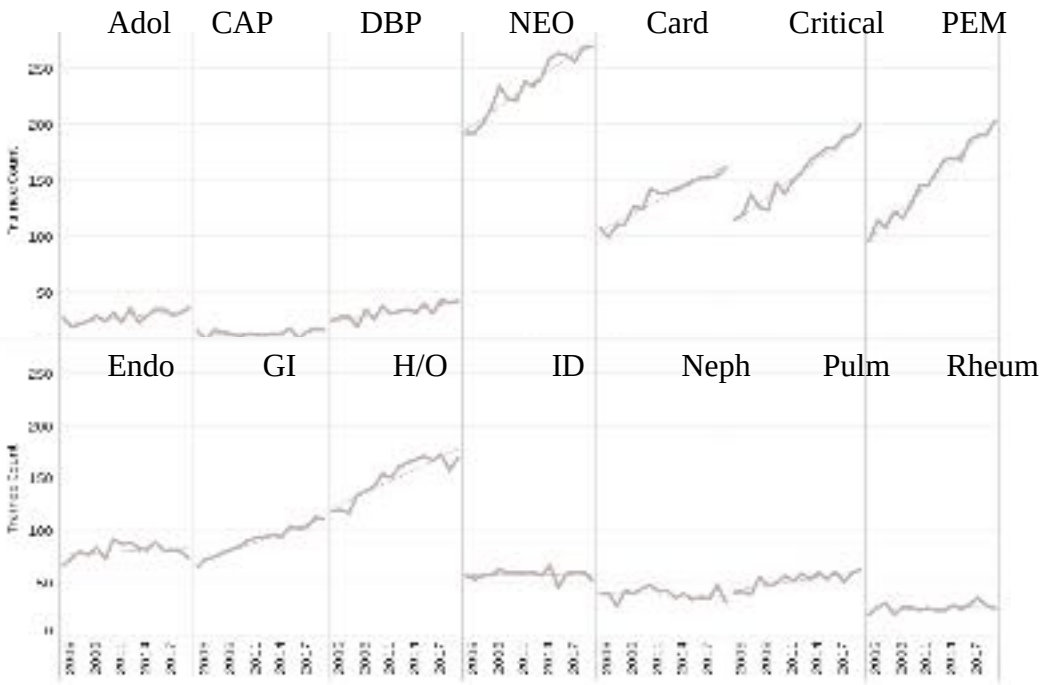




THE MATCH

* MD, DO, IMG

Growth in ABP Subspecialties 2000-2020



https://www.abp.org/content/subspecialty-fellowship-statistics

National Pediatric Workforce: Future Initiatives

The Pediatric Workforce: Recent Data Trends, Questions, and Challenges for the Future

PEDIATRICS Volume 147, Issue 6, June 2021:e2020013292

Pediatrics 2025: The AMSPDC Workforce Initiative

Robert J. Vinci, MD[1], Laura Degnon, CAE[2], and Sherri U. Devisser, MD[3]

https://doi.org/10.1016/j.jpeds.2021.02.004

Domain I: Attract diverse trainees into undersubscribed subspecialties

Domain II: Workforce data/needs and access

Domain III: Economic strategy

Domain: IV: Other considerations for attracting high quality medical students into pediatrics

Six Megatrends in Pediatric Health Care

Population health and health equity

Genomics

Treatment of rare diseases by gene therapy

Behavioral and mental health

New diseases, undesirable trends and unplanned events

The pediatric subspecialty workforce

Six Top Targets - Pediatric Nephrology Research

AKI in Developing World

Environmental toxins – prenatal and postnatal kidney health

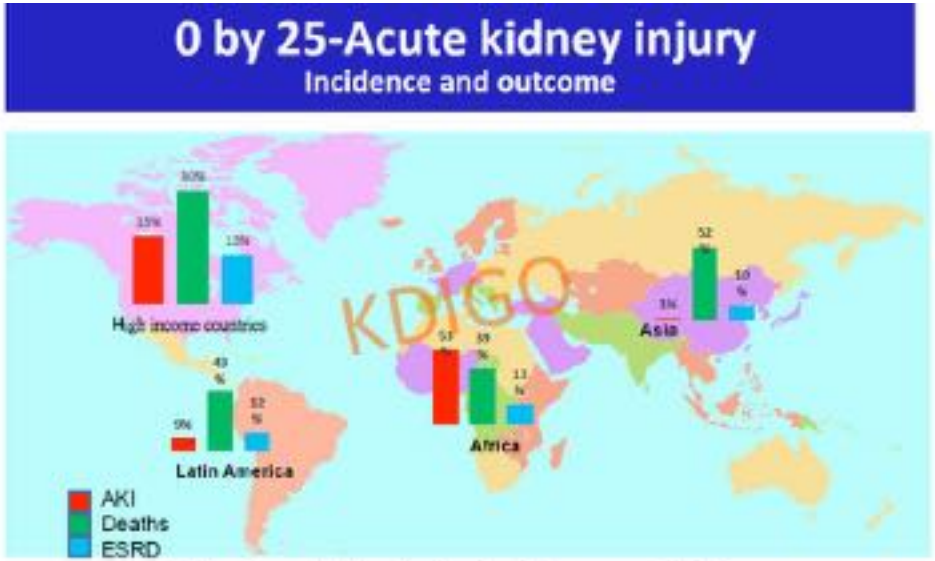
Perinatal Health

UTI (Early Diagnosis/Prevention)

Cardiovascular-Renal Disorders

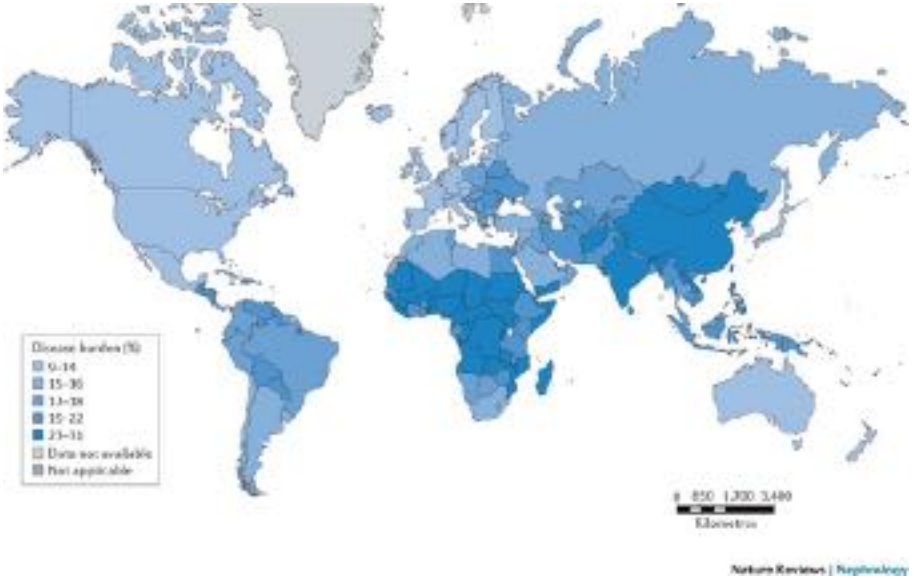
Social Determinants of Health

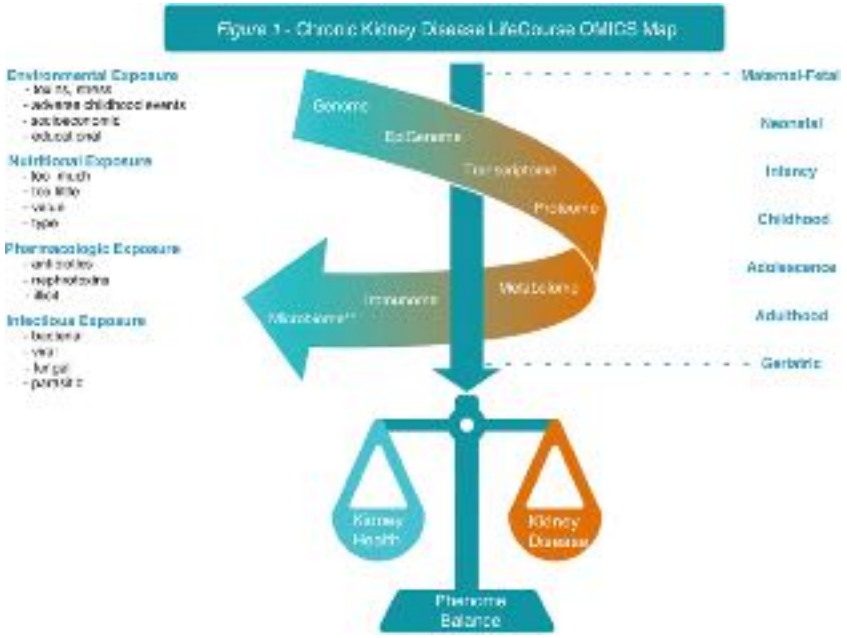
AKI in Developing World



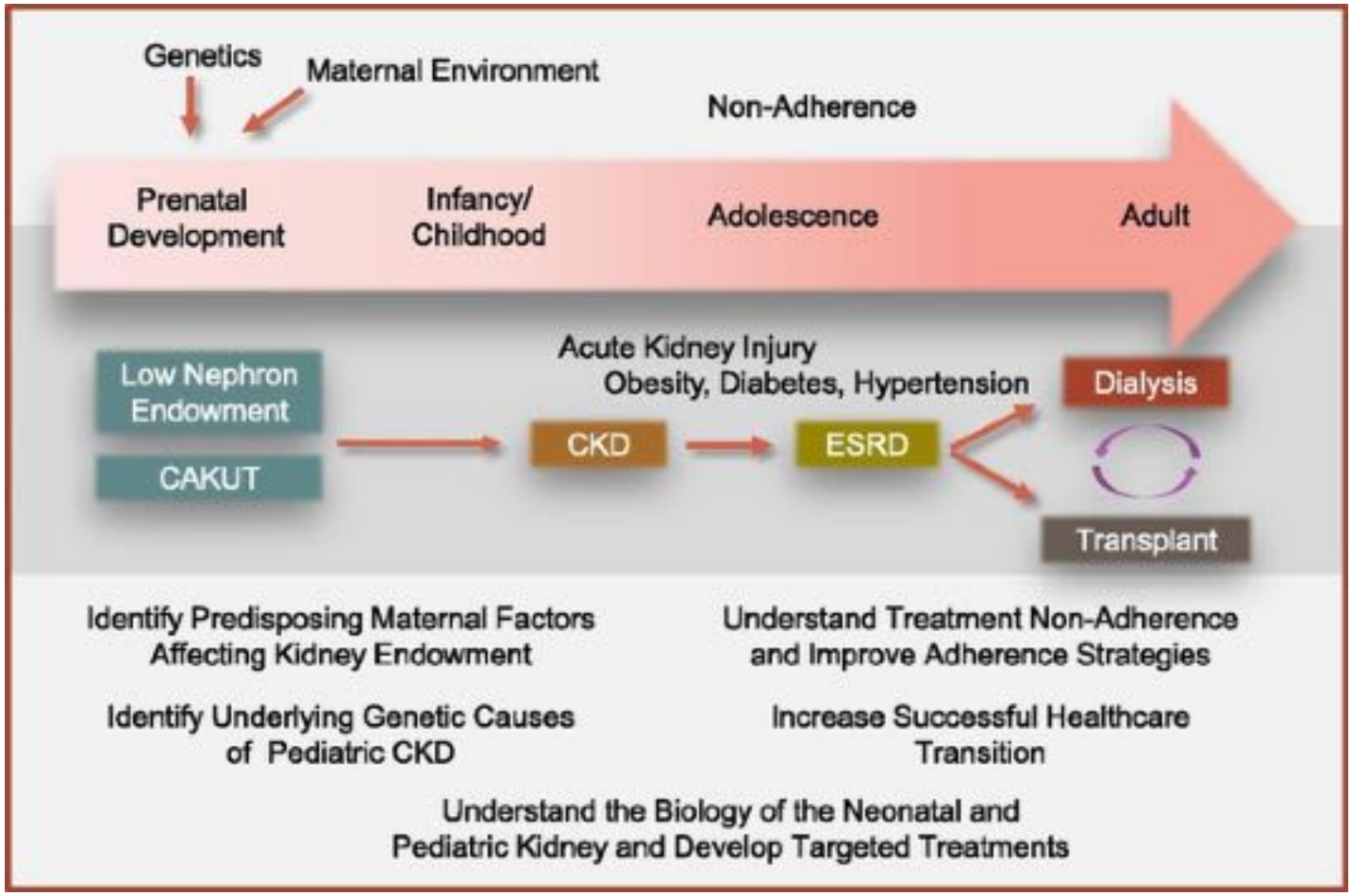
Global variation in the incidence of AKI

Environmental Toxins – Prenatal and Postnatal Kidney Health

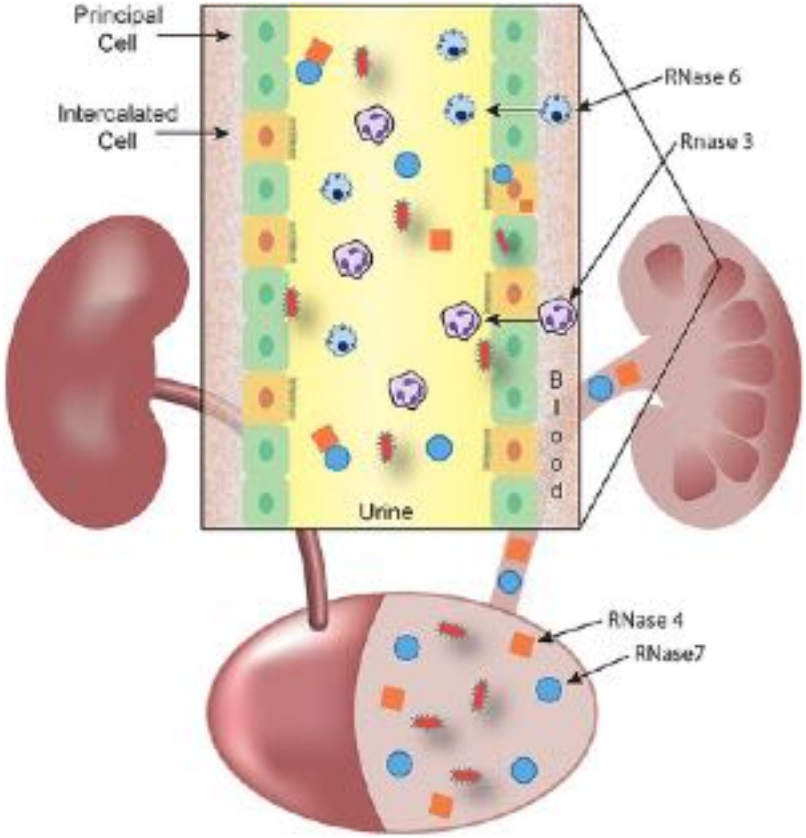


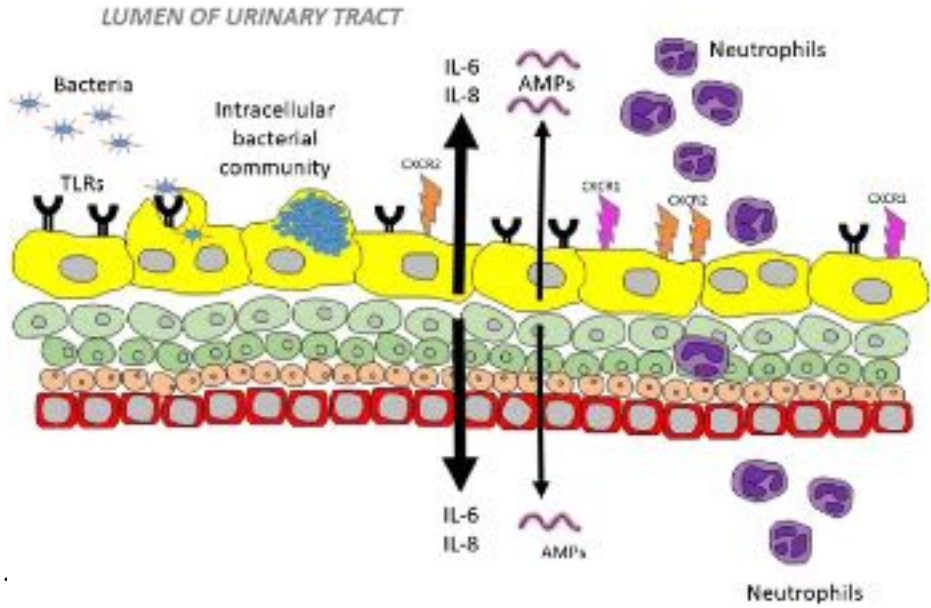


Perinatal Health

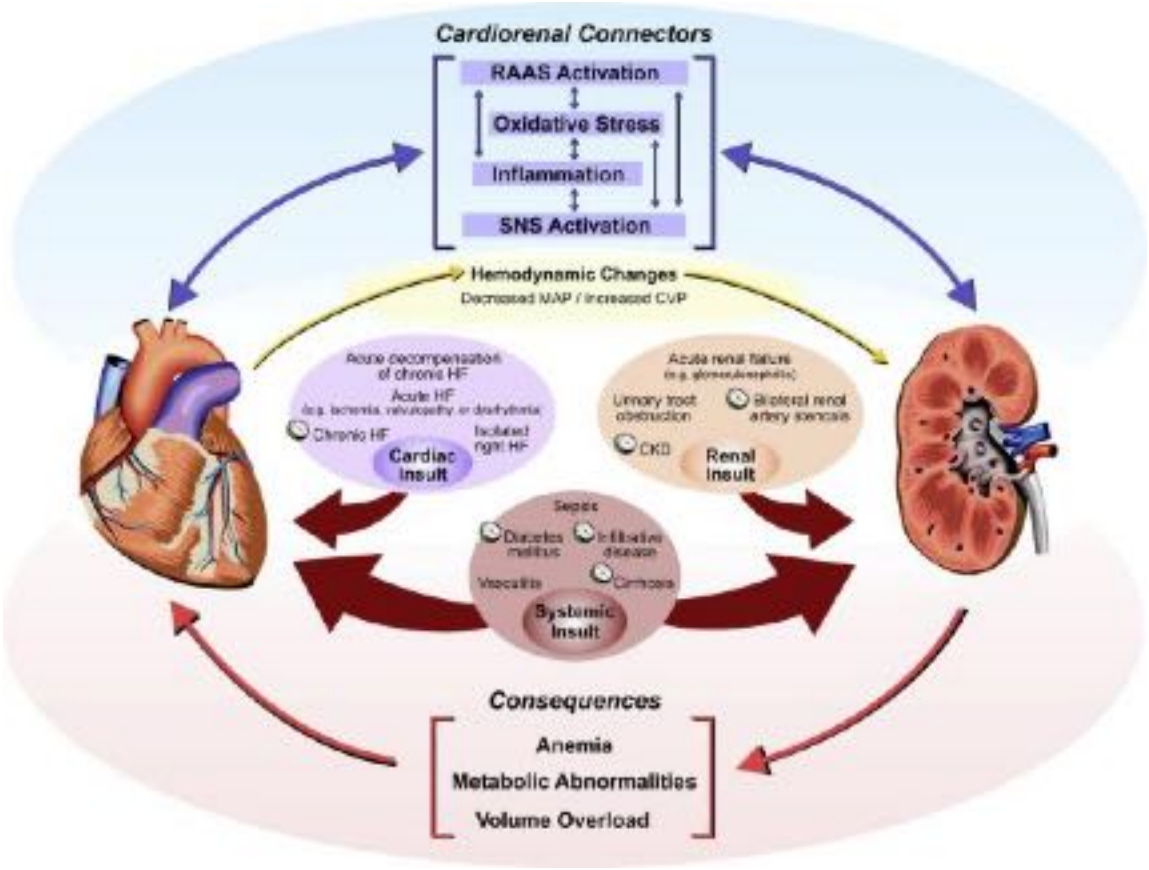


UTI (Early Diagnosis/Prevention)

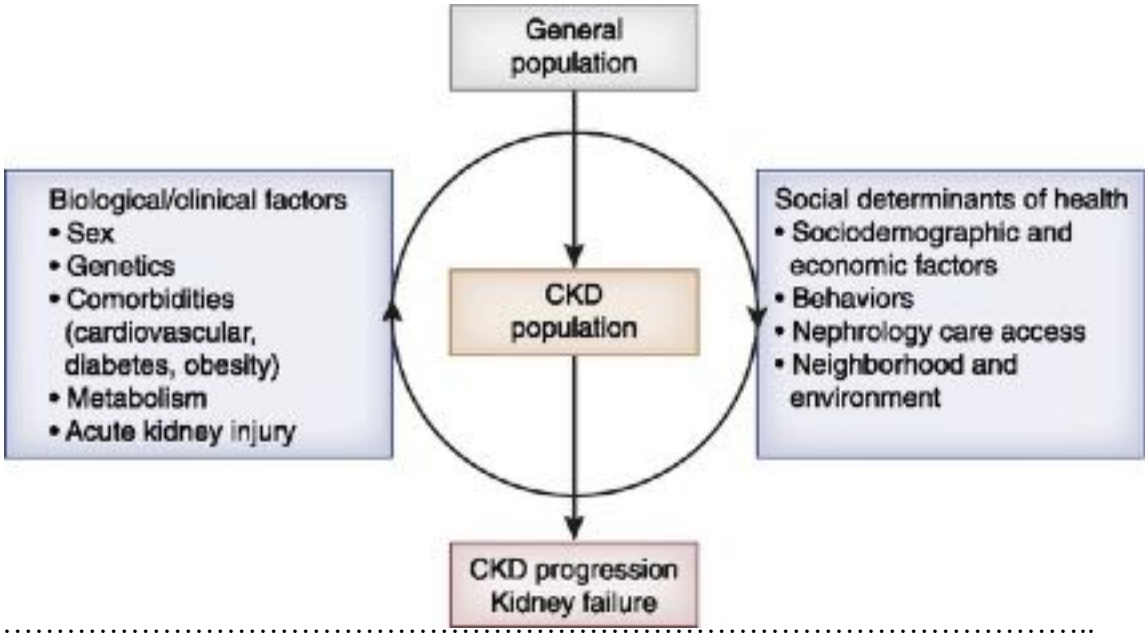




Cardiovascular-Renal Syndromes



Social Determinants of Health



Key Research Approaches in Pediatric Nephrology – Consider Where You Might Join In!

Disease Registries – local, international

Observational Studies

Collaborative (Team) Research

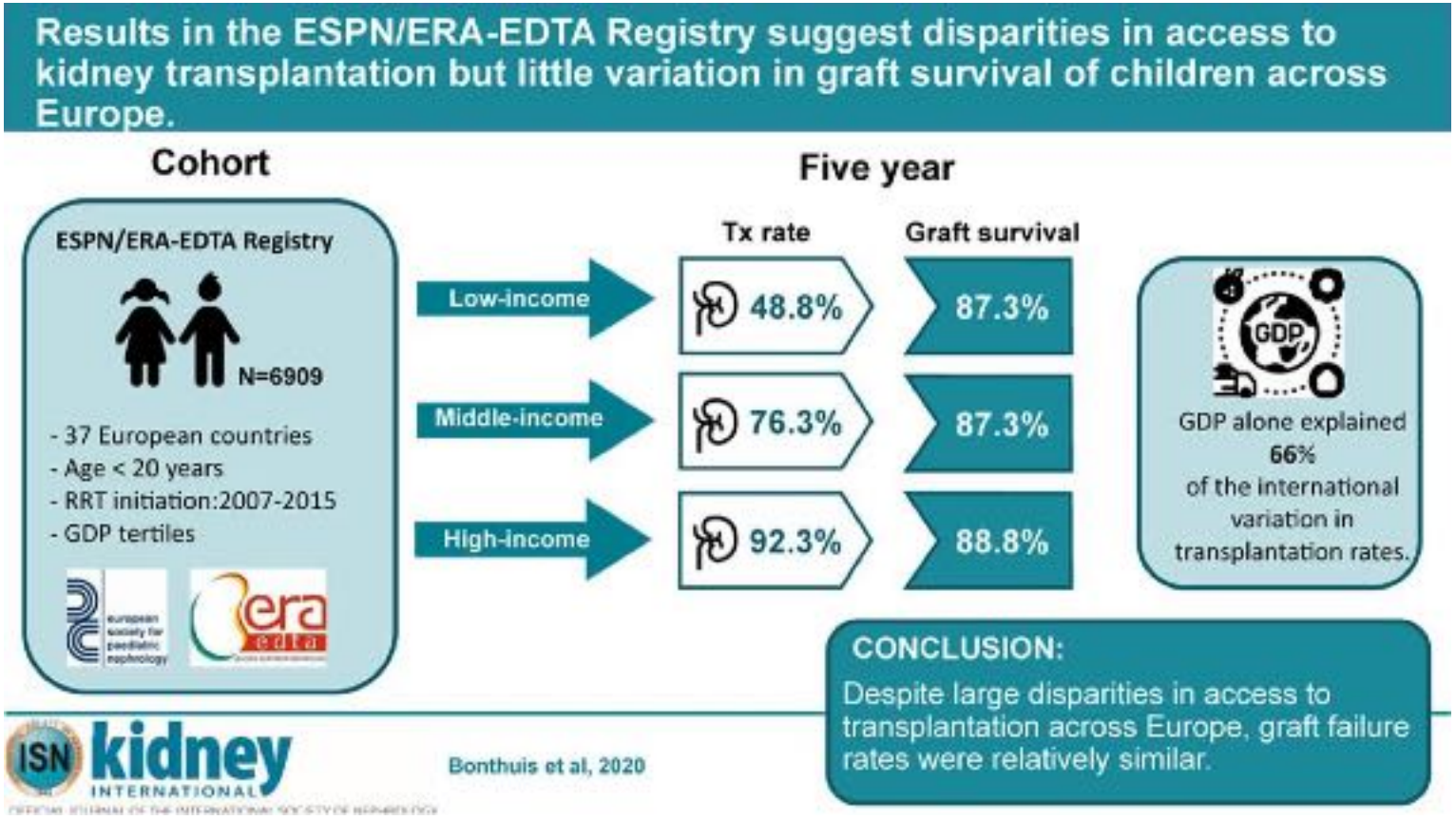
- Bench Research
- Translational Research

Large Data Sets

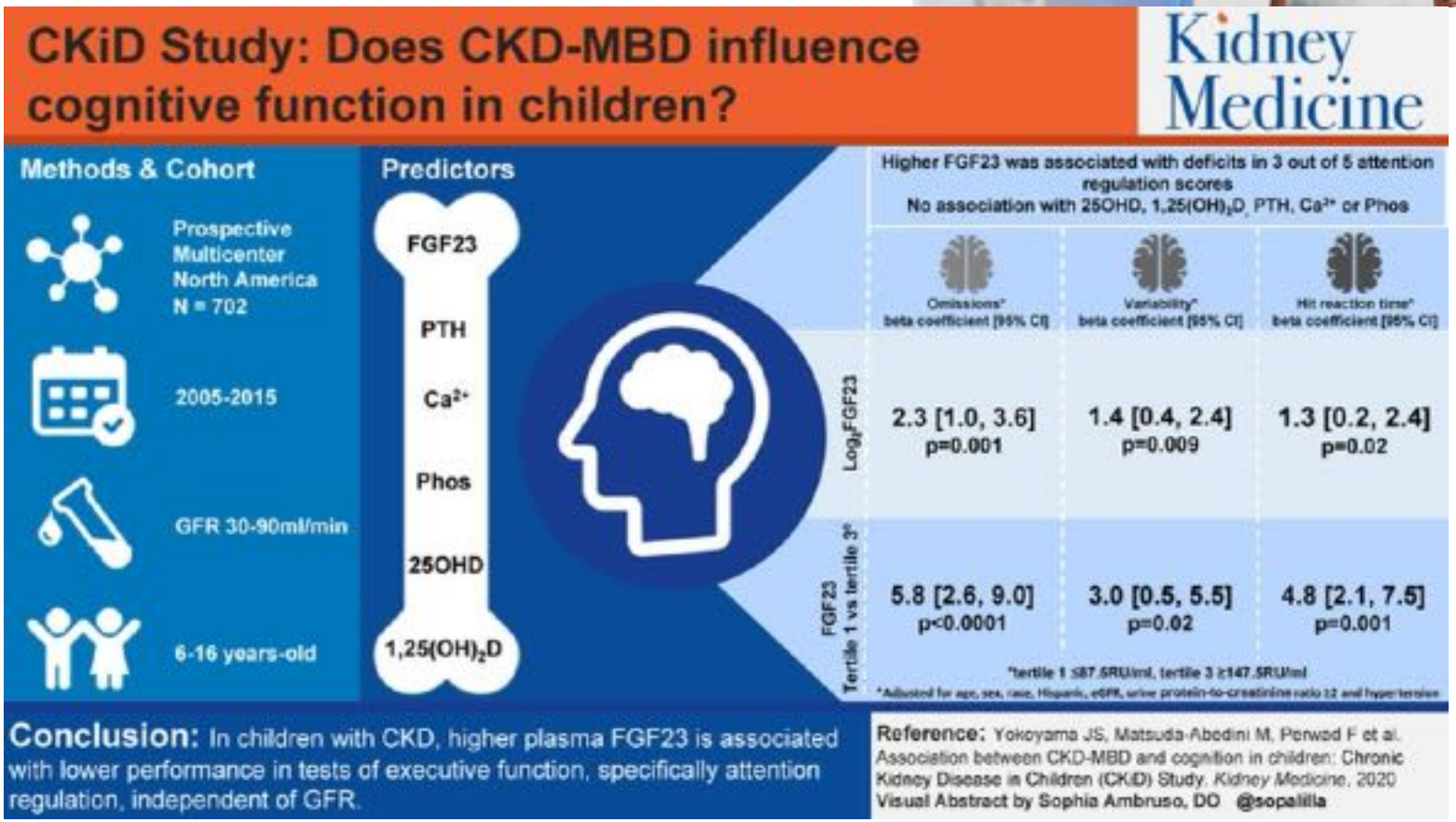
High Quality Interventional Trials

Disease Registries – Local, International

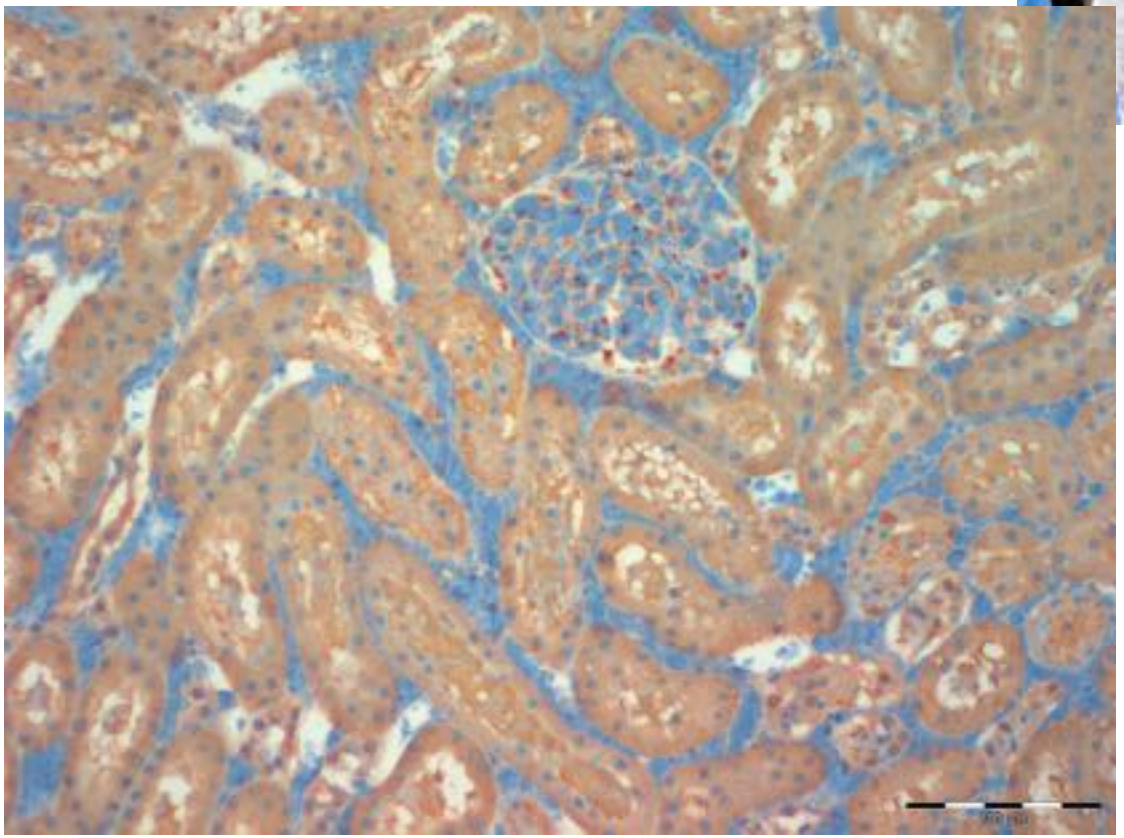
ESPN/ERA-EDTA Registry



Observational Studies



Collaborative (Team) Research - Bench Research



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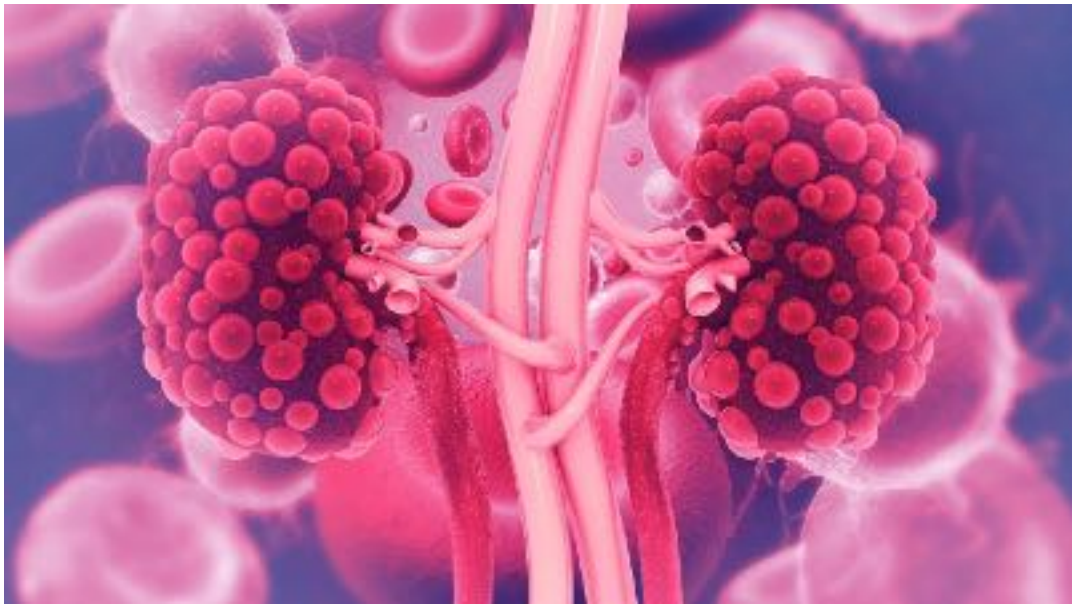
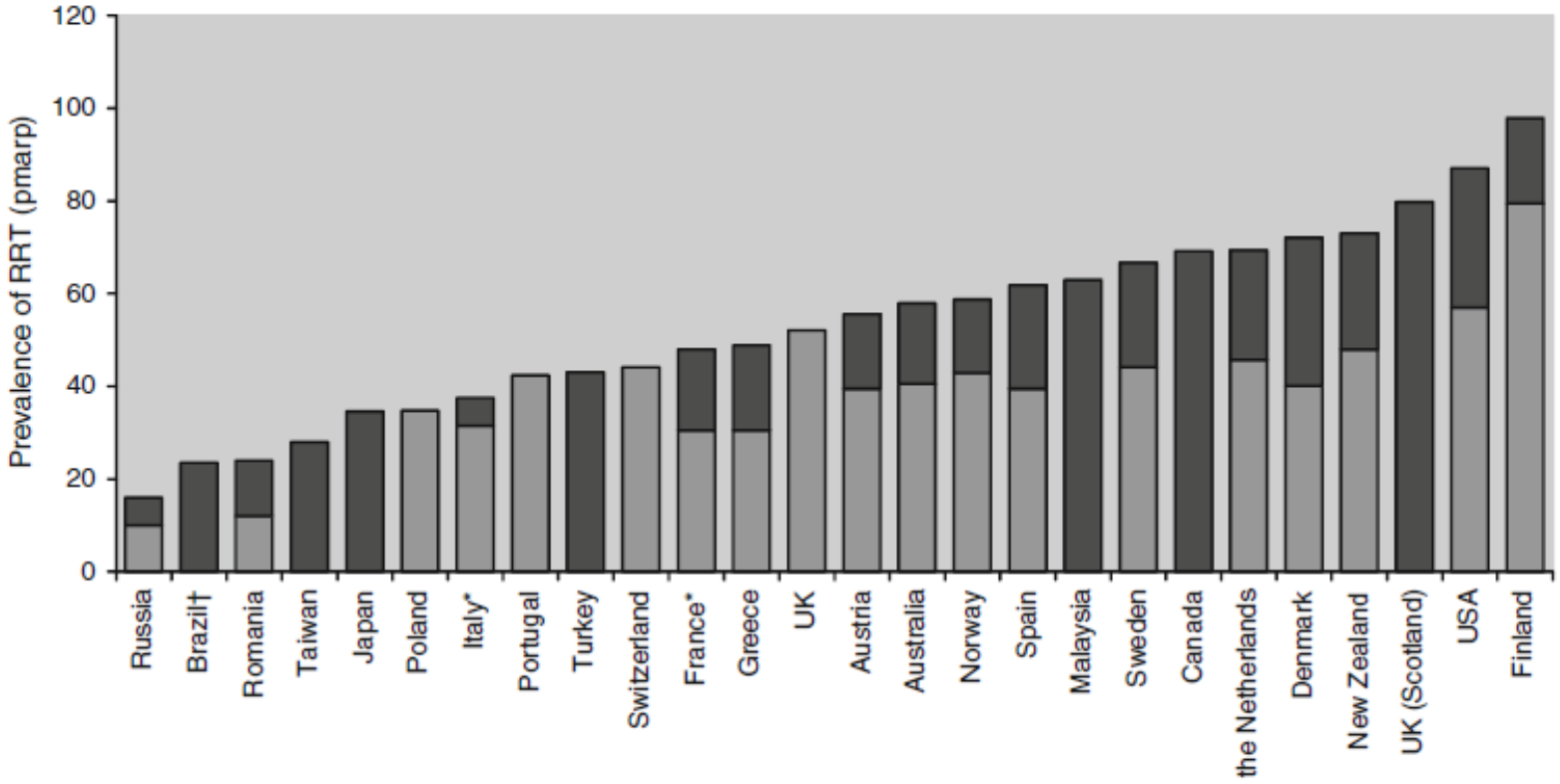
Medic BioMed Res Int 2015

Collaborative (Team) Research - Translational Research



Guy-Woodford

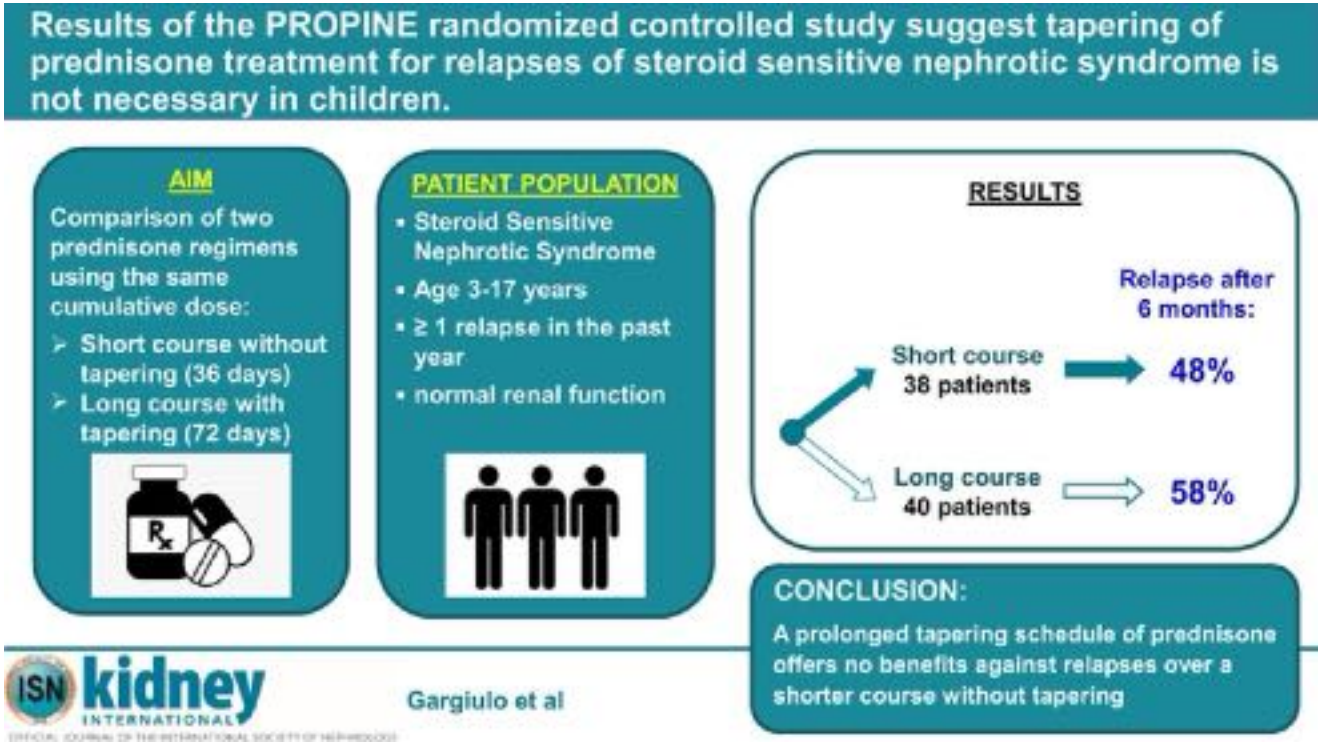
Large Data Sets



Harambat Ped Neph 2012

High Quality Interventional Trials

Answering important clinical questions



Improving Research in Pediatric Nephrology

How can **YOU** get involved in pediatric nephrology research and contribute?

_____

_____

_____

Key Points

1. 6 megatrends in child health should drive research priorities in the future
2. 6 top targets for Pediatric Nephrology research in the near future include AKI, Environmental toxins, Perinatal Health, UTI, CV-Renal Disorders, Social Determinants of Health
3. There are many ways to contribute to research in Pediatric Nephrology – join in!!

Thanks

Nationwide Children's Hospital

Fans of IPNA and AfSPN