



ACCRF Overview

Nicole (Nikki) Spardy Burr, PhD, Scientific Program Officer

The Roots of ACCRF



ACCRF was founded by Marnie and Jeff Kaufman. Marnie was diagnosed with ACC at 38 years old when she had four boys under the age of 10.

ACCRF is a public charity established in December 2005 in Needham, Massachusetts, USA



ACCRF Overview

MISSION

Accelerate the development of **better treatments and a cure** for ACC patients

GOAL

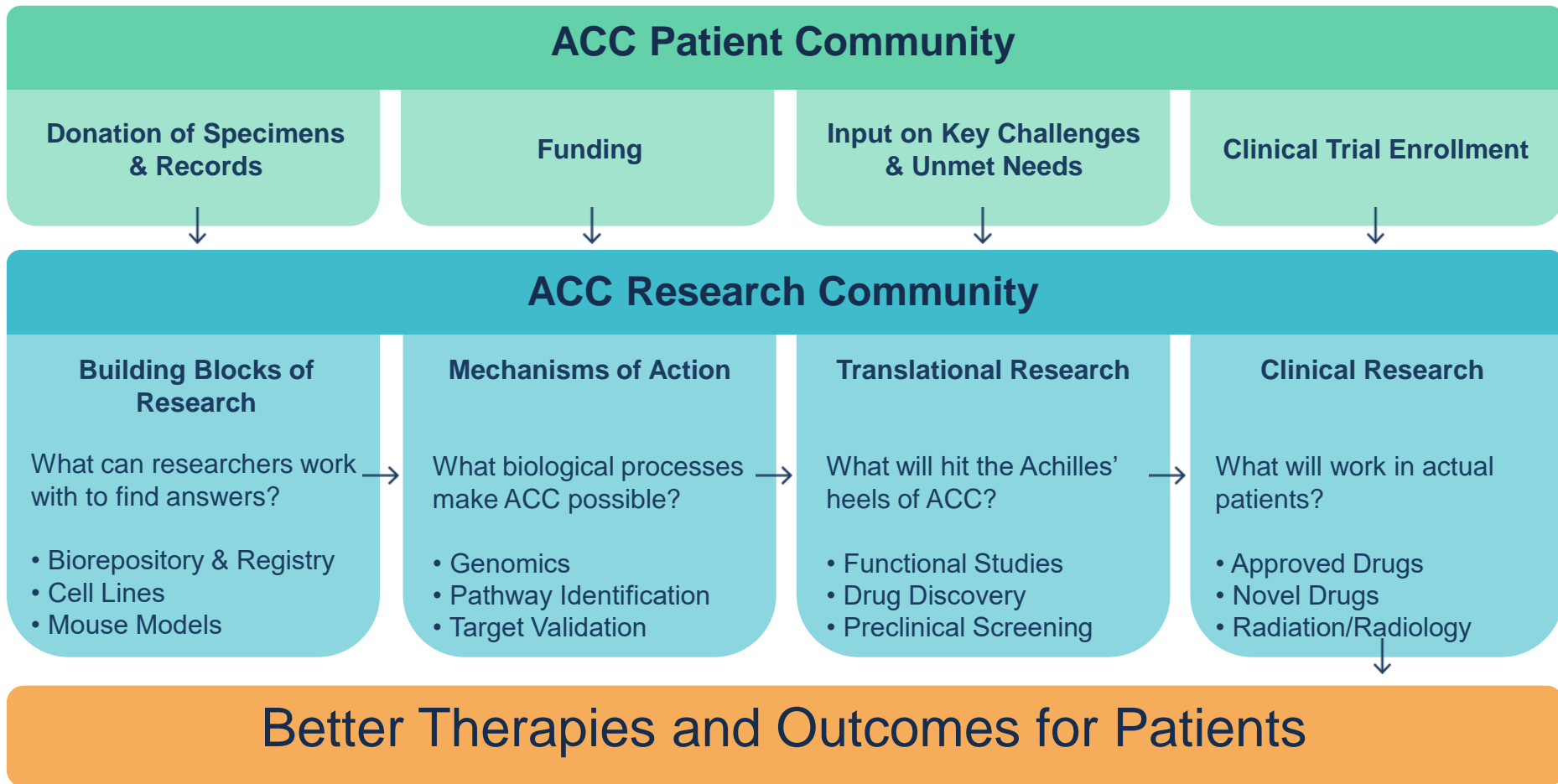
Develop a **pipeline of clinical trials** based on the best available science

STRATEGY

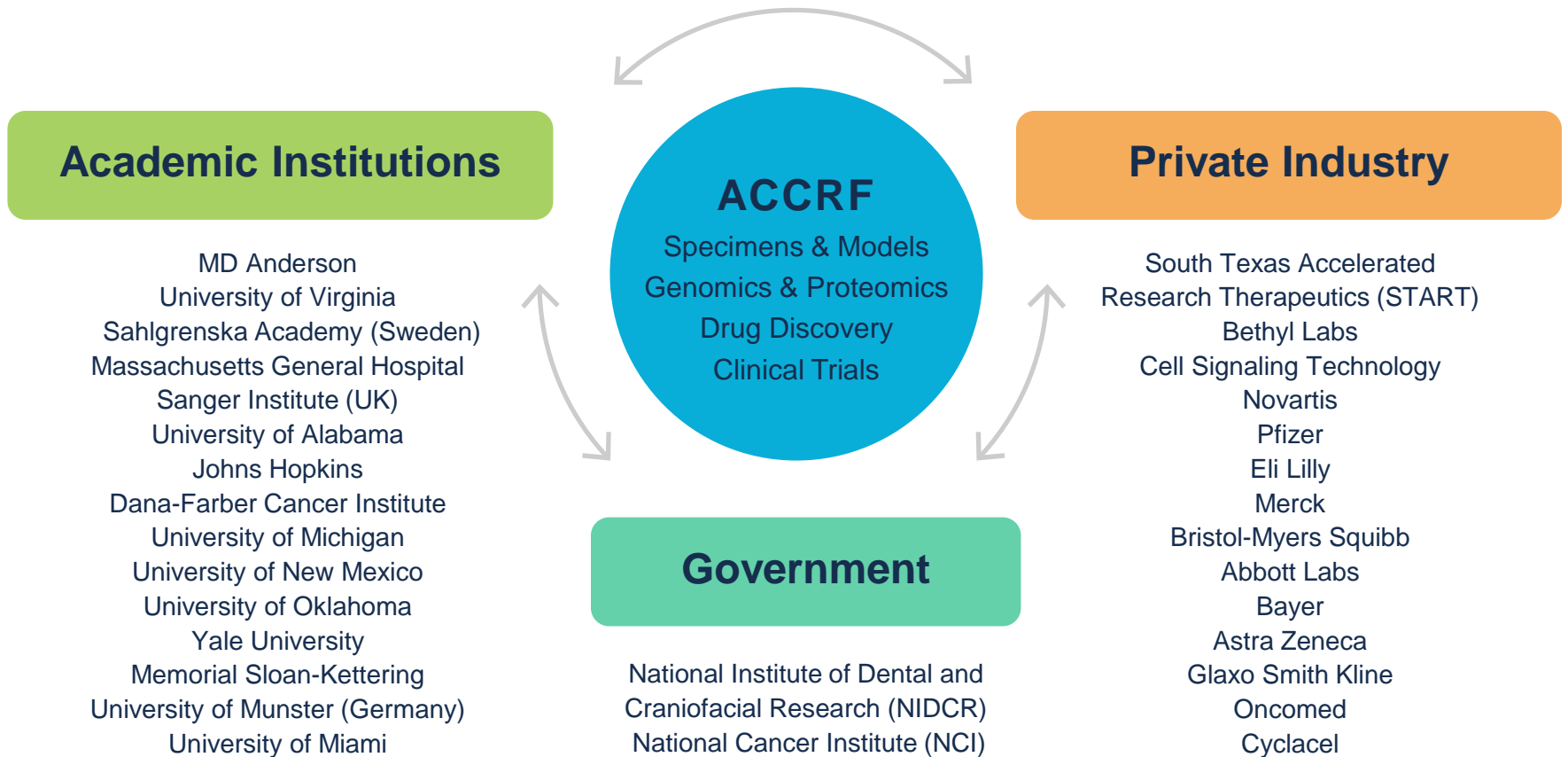
Create a **community of researchers** following a **coordinated plan** that is driven by **supportive and supported patients**



ACCRF Research Agenda



ACCRF Research Network

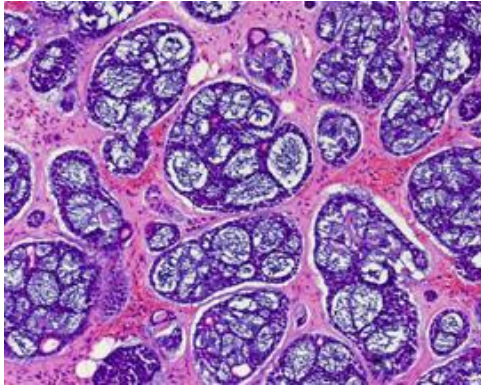


ACComplishments

	2005	2018
Biobanking	Limited	Repositories with hundreds of frozen tumor specimens
Cell Lines	Multiple invalid models	Misidentifications discovered; valid models in development
Animal Models	None	20+ mouse xenografts developed; first transgenic models
Genomics	Sporadic reports of translocations	<ul style="list-style-type: none"> • Discovery of recurrent t(6;9) and MYB-NFIB fusion gene • Identification of additional molecular targets with potential therapies: NOTCH, FGFR, IGF-1R, HDAC
Preclinical Drug Screens	None in valid models	<ul style="list-style-type: none"> • Open xenograft platform for academia and industry • Strong relationships with biopharmaceutical companies • 100 anti-cancer compounds screened in xenografts
Mobilizing Patients	Limited	Tissue donations, clinical trial accrual and \$15 million in donations
NIH Commitments	Negligible	Over \$25MM for salivary gland tumor research (NIDCR)
Clinical Trials	Few, small & haphazard	Multiple science-driven trials with improved designs, enrollment, data quality and patient outcomes

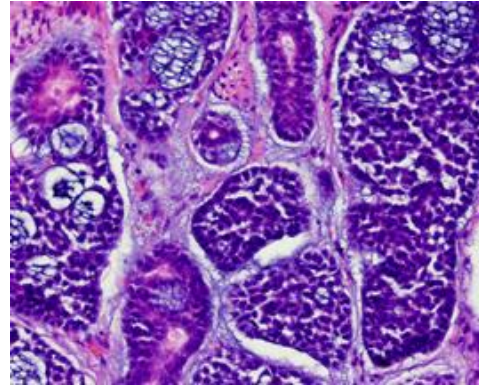


How we think ACC works



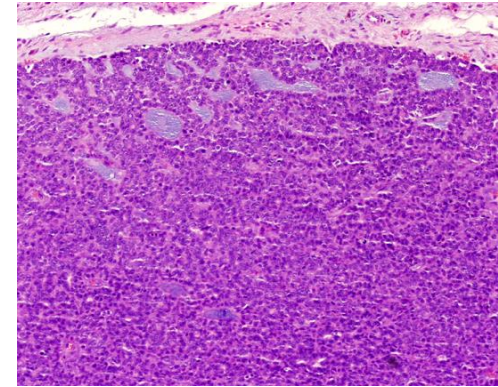
Grade 1
No solid component

MYB/MYBL1
fusion or overexpression
(90-95% of cases)



Grade 2
<30% solid

Secondary alterations in other genes
(*NOTCH1*, *FGFR*, *IGF*, *PI3K* and chromatin modifiers)
drive disease progression



Grade 3
>30% solid

Therapies: Research grants focused on finding **MYB/L1 inhibitors**

NOTCH inhibitors show early signal in NOTCH-mutant ACCs
Clinical trials are investigating other **targeted and immune therapies** in ACC



ACCRF Funds in Action

ACCRF grants to the University of Virginia...

- Jump-started mouse model development
- Blossomed into genomic studies and preclinical drug screening, and
- Led directly to the first science-driven clinical trials of targeted drugs in ACC patients



Dr. Christopher Moskaluk
University of Virginia



Dr. Patrick Dillon
University of Virginia



ACCRF Funds in Action

ACCRF grants to MD Anderson...

- Jump-started tumor banking that eventually gained NIH funding
- Blossomed into the identification of drug targets in aggressive cases of ACC, and
- Is leading to the development of clinical trials for ACC patients with NOTCH-altered tumors



Dr. Adel El-Naggar
MD Anderson



Dr. Renata Ferrarotto
MD Anderson



ACCRF Funds in Action

ACCRF grants to Dana-Farber Cancer Institute...

- Jump-started immunologic profiling of ACC tumors
- Blossomed into the identification of PD-1 and PD-L2 markers expressed in ACC, and
- Is leading to clinical trials for ACC patients with PD-1 inhibitors in combination with radiation, chemotherapy and targeted drugs.



Dr. Glenn Dranoff
DFCI, Novartis



Dr. Jon Schoenfeld
DFCI



Dr. Nicole Chau
DFCI



ACC Targeting Approaches

- Multikinase (FGFR, VEGFR) inhibitors
- NOTCH inhibitors
- Immune checkpoint inhibitor combinations with radiation, chemo
- MYB inhibitors and DNA vaccine
- MDM2 inhibitors
- HDAC inhibitor combinations
- PSMA radiopharmaceuticals
- ...and more in the pipeline...



Keep yourself updated...

- Sign up to receive ACCRF research updates via email...

Adenoid Cystic Carcinoma Research Foundation

SEARCH

GET OUR NEWSLETTER

DONATE NOW

HOME LIVING WITH ACC TREATMENT OPTIONS RESEARCH TAKE ACTION ABOUT ACCRF CONTACT US

THE DOHERTY & PALUS FAMILIES
Golf can be a good walk for a good cause. This is the lesson to be learned from the Doherty and Palus families. *CJ Doherty, Inc. Golf Outing to Benefit ACCRF*

**Hope is a Powerful Ally.
And Research is the Angel of Hope.**

Adenoid cystic carcinoma (ACC) is a rare cancer of secretory glands, typically originating in the head and neck region.

The Adenoid Cystic Carcinoma Research Foundation (ACCRF) supports research into adenoid cystic carcinoma that will accelerate the development of improved therapies and a cure for the disease. [Read More...](#)

- Check the “Clinical trial- current studies” section on our website!



Summary

- ACCRF has jump-started the field of ACC research through:
 - World-class Scientific Advisory Board driving a directed agenda
 - Creation of biobanks, preclinical models and research network
 - Target discovery and validation leading to clinical trials
- ACCRF is prioritizing therapy discovery and innovative clinical trials, with several promising concepts in development
- We ask for your support to achieve our goal of having the **first FDA-approved therapy for ACC by 2020**





Adel El-Naggar



Chris Moskaluk



Göran Stenman



Andy Futreal



Michael Wick

Thanks to ACC Research Heroes!



David Sidransky



Lillian Siu



Bruce Chabner



Robert Haddad



Ned Sharpless



Gigi Lozano



Irwin & Joan Jacobs

*ACCE*lerate the *CURE*