

PANCREATIC
CANCER
ACTION
NETWORK

Accelerating Pancreatic Cancer Clinical Development

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President and CEO

Precision Promise

**A new model for rapidly accelerating
therapeutic development in pancreatic
cancer partnering with the FDA**

The World's Toughest Cancer

3rd
Leading
cause of
cancer
death



1250 people
will be
diagnosed
everyday
worldwide

**Pancreatic cancer on the rise.
The need is urgent!**

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6

Challenges

#1

Not enough researchers or resources



#2



Patients are diagnosed too late

81% of patients are diagnosed
once their disease has spread

#3

Clinical trial enrollment rate too low

**Only 4.2% of pancreatic
cancer patients enroll in
clinical trials**

#4



Traditional trials aren't rapidly producing new treatment options, making this a risky drug development space

#5

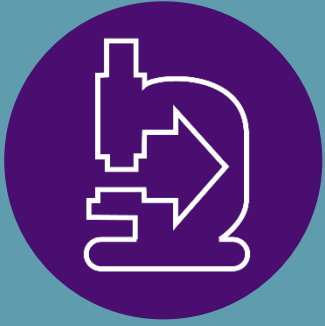


**There is insufficient knowledge of
the disease and how best to treat it**

#6



**Not enough people know about
the disease and PanCAN**



Research and Clinical Initiatives

We pioneer and push science forward through our grants program, our own clinical studies and cultivation of the research community.



Government Advocacy

We demand federal funding for research and advocate for legislative support on Capitol Hill.



Patient Services

We connect patients with the latest treatment options and provide free, in-depth disease information.



Community Engagement

We lead and mobilize communities across the globe to raise awareness and funds to fight the disease.

Cumulative Research Investment

\$105 million



Peer-Reviewed
Grants



Know Your
Tumor



Precision
Promise®



Early Detection
Initiative



Patient
Registry

We Help Patients Get on the “Right Track”



Right
Team



Right
Tests



Right
Treatment



Share At
Every Step

Patient Services Annual Numbers

13,450
new patient
contacts

14,250
clinical
trial
searches

41,000
interactions

58,000
booklets distributed

64% increase in new patient
contacts over last year

Be **BOLD**

Six Challenges

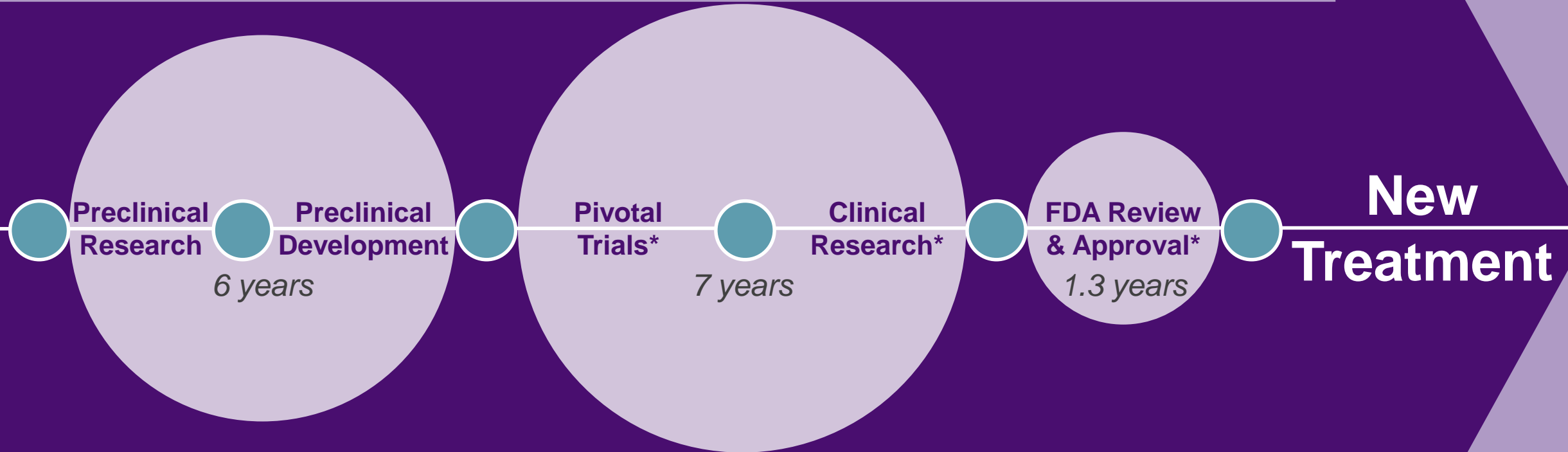
1. Not enough researchers or resources
2. Patients are diagnosed too late
- 3. Clinical enrollment rate too low**
- 4. Traditional trials aren't rapidly producing new treatment options, making this a risky drug development space**
- 5. There is insufficient knowledge of the disease and how to treat it**
6. Not enough people know about the disease and PanCAN

Lack of preliminary evidence has led to high failure rates in late-stage development

- Analysis looking at pancreatic cancer trials from 1995-2015 published in JAMA Onc in 2016:
 - showed that historic failures were largely due to insufficient evidence going into Phase III
 - Out of 24 phase II/phase III pairs (1995-2015), 10 trials continued despite not meeting the primary end point for the phase II trial
 - Only a 10% success rate in Phase III over this period of time
 - ***11,571** patients could have been re-allocated

Drug Development Takes Time and Money

We must influence smart, efficient and cost effective trials



Average development time: 14-15 years

Average cost: approximately \$2.5 billion

Patients Tell Us

- 1) Patients and their families want more patient-centric approaches
- 2) Patients want options
- 3) Patients are frustrated by clinical trial barriers such as slow drug to market progression and the recruitment process
- 4) Most patients want to do anything they can to survive
- 5) Many patients are willing to undergo tests to help themselves and to help others in the future
- 6) Patients are excited about an innovative trial design such as crossing over into other arms and novel treatment options

Large-Scale Research Initiatives



**Know Your
Tumor[®]**



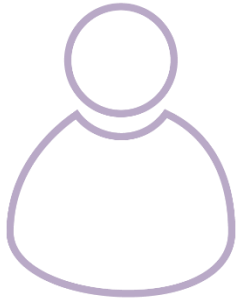
**Precision
PromiseSM**



**Early Detection
Initiative**

Know Your Tumor[®]

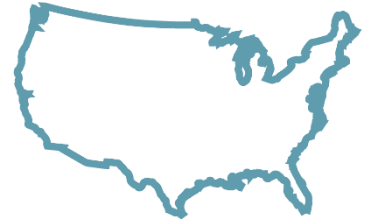
*A pioneering precision medicine service that
may lead to better treatment options.*



96%
successful
biopsies



32%
from community
physicians



1933
patients
enrolled



1134
reports
completed



49
states with
patients enrolled

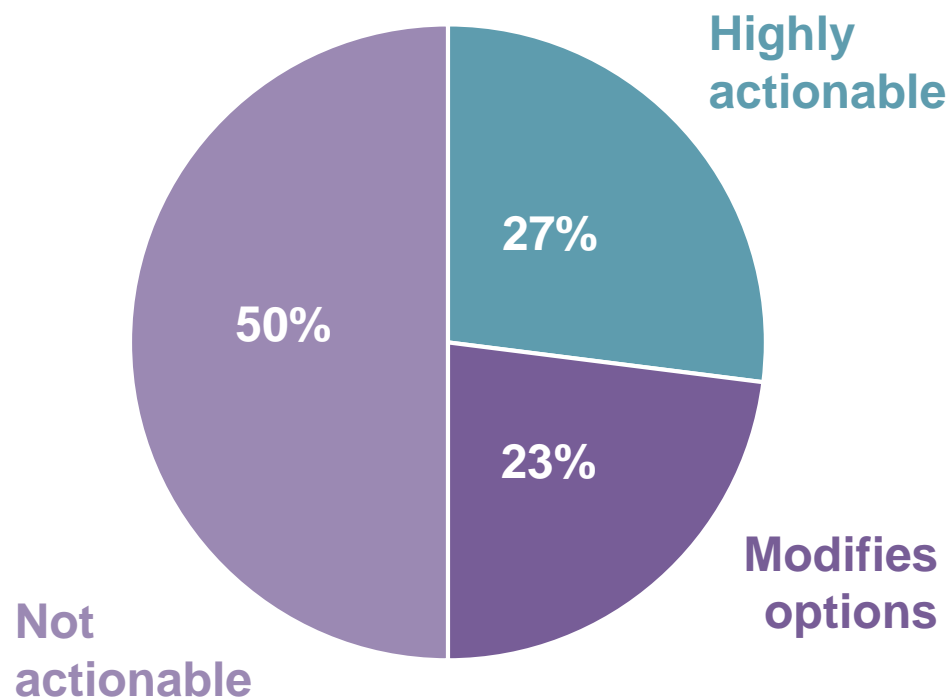
Know Your Tumor[®]

June 2014 – June 2019

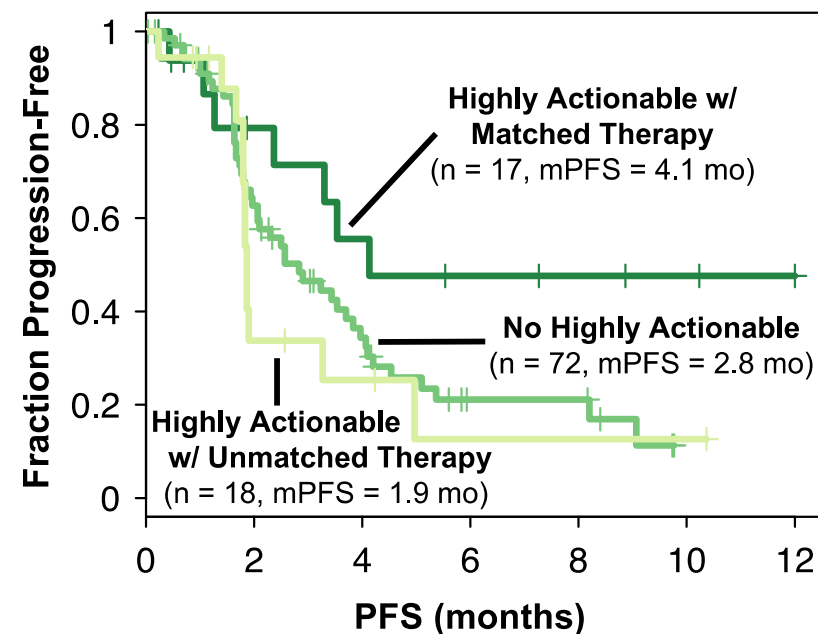
Molecular profiling of pancreatic cancer patients: Initial results from Know Your Tumor

Tumor DNA Sequencing

June 2014 – June 2017, 640 reports



Patients with highly actionable alterations who received matched therapy had improved survival over those that did not receive matched therapy, $p=0.03$



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**Precision Promise:
an innovative model for
accelerating new
treatment options**

**Precision PromiseSM
is an adaptive
randomized phase II/III
registration ready
clinical trial platform**

- PanCAN holds the IND
- Master Protocol with two control arms and multiple investigational arms
- Initially for 1st and 2nd line metastatic pancreatic cancer patients
- Rerandomization option for 1st line patients
- Primary endpoint is overall survival
- 175 patients for drug approval

Molecular testing and biomarker exploration across all patients

- Tissue and blood collected on all patients at baseline, on-treatment, and post-treatment
- Uniform molecular testing at baseline and 8 weeks post-treatment including DNA and transcriptome assays
- Opportunity for arm-specific biomarker mining to define mechanism

Investigating Supportive Care Best Practices

1. Patient Reported Outcome (PRO) questionnaires

2. Actigraphy sub-study

- Changes in activity may be an early marker for disease progression

3. Body composition analysis

- Opportunity to correlate muscle and fat loss with tumor inflammation features and response to treatment

4. Enzyme replacement therapy

- Analysis of correlations between the use of enzymes, improved quality of life and overall survival

Precision Promise Value Proposition to Industry

- 1. *Steadfast FDA Support*** – FDA leadership and designated FDA liaison extremely supportive since onset of planning
- 2. *Established Infrastructure*** – 14 clinical trial sites, 100 pancreatic cancer experts across all governing Committees, operational vendors and partners in place
- 3. *Lower Cost*** – Estimated 30-50% savings over traditional Phase II/III oncology registration trials
- 4. *Accelerated Time to Market*** – Reduced trial start-up time and seamless transition from Phase II to Phase III
- 5. *Fewer Patients*** – Adaptive design requires only 175 patients for Phase II/III registration

A Collaboration of Leading Pancreatic Cancer Experts

Operations

IND holder:



CRO:



Statistical
design:



Genomics
sequencing
and analysis:



Infrastructure

Steering Committee

Diane Simeone, MD (Chair)
Sudheer Doss, PhD
Andrew Hendifar, MD
Manuel Hidalgo, MD, PhD

Anirban Maitra, MBBS
Victoria Manax, MD
Lynn Matrisian, PhD, MBA
Vincent Picozzi, MD

Design

Biomarker

Supportive
care

Arms
selection

Operations

Clinical
trial
consortium

Pharma

Current Industry Members



Founding Precision Promise Members



Trial Sites



PanCAN Research Publications

Clinical Trials Analysis

- Trial benchmarks of success – *JAMA Oncology* 2016
- Trial landscape updates – *ASCO Educational Book* 2016
- Trial landscape – *J Clinical Oncology* 2013

Know Your Tumor® Molecular Profiling

- Outcomes of first 640 patients – *Clinical Cancer Research* 2018
- Case study with IDH1 mutation – *Cancer Biology & Therapy* 2016
- Liquid biopsy pilot study – *Oncotarget* 2016
- Metastatic vs. primary tumors – *British J. Cancer* 2019, in press
- DNA damage response pathways – *JCO Precision Oncology* 2019, submitted

Supportive Care & Patient/Caregiver Surveys

- Pancreatic cancer enzyme replacement – *Pancreas* 2019
- Pancreatic cancer-associated weight loss – *The Oncologist* 2018
- Patient and caregiver perceptions – *Pancreatology* 2015
- Awareness of treatments and trials – *J Gastrointestinal Oncology* 2015

2020 Goal

- Private vs. public funding – *Gastroenterology* 2017
- Projecting cancer incidence & death – *Cancer Research* 2014
- Current landscape in Europe, North America and Asia – *Oncology & Hematology Review* 2019, in press



SURVIVOR

Part of the solution: Know your cancer: and it.

I am here in honor of:

Myself!

6 1/2 year Survivor
PANCREATIC CANCER ACTION NETWORK
ADVANCE RESEARCH. SUPPORT PATIENTS. CREATE HOPE.
www.pancan.org

CHECK-UP
SURVIVORS

CHECK-UP

CALL-IN
VOLUNTEERS

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