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CORAL GABLES, FLORIDA | 2019

Understanding Clinical Trials

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Overview

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Clinical Trials Basics

- What are clinical trials
- How are clinical trial designed
- How is safety monitored in clinical trials
- What myths exist about clinical trials
- How to find clinical trials

Open and Enrolling Pancreatic Cancer Clinical Trials

Clinical Perspective on Clinical Trials

- Enrollment process
- Patient follow up

Clinical Trial Basics

What are Clinical Trials

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- Research studies testing new treatments and screening or prevention tools for a particular disease
- Play an important role in the development of new treatment options and screening tools for a disease
- Necessary to determine whether new treatments or screening tools developed in the laboratory are beneficial to people
- Should be considered at diagnosis and during every treatment decision

Phases of Clinical Trials

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Phase 1:

- Determine safe dose
- Monitor side effects
- Question being answered: Is it safe?

Phase 2:

- Pancreatic cancer-specific, effectiveness
- Question being answered: Does it work?

Phase 3:

- New treatment compared to standard of care
- Question being answered: Is it better?

Clinical Trial Designs

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- Number of arms or treatment groups depends of the design of the clinical trial
  - Single arm
  - Multiple arms
- Blinding helps eliminate any bias the doctors and/or patients may have toward a particular treatment
  - Double-blind
  - Single-blind
  - Open-label
- Clinical trials with multiple arms, may also be randomized, meaning that patients will be randomly assigned to the different arms

Emerging Clinical Trial Designs

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Newer clinical trial designs are emerging and include:

- Basket Trials
- Umbrella Trials
- Platform Trials

Clinical Trial Eligibility

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- Eligibility criteria is developed for each clinical trial to:
  - Identify the patient population with enough in common to be able to determine whether or not the treatment helped
  - Protect patients and ensure that coexisting medical conditions that could put the patient at risk on the trial are not allowed
- Standard eligibility requirements include:
  - Type and stage of cancer
  - Prior treatments received
  - Age
  - Medical history
  - Current medical condition

Clinical Trial Endpoints

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- Clinical endpoints or specific measures of a clinical trial's impact are defined in the clinical trial protocol
- Clinical endpoints may include:
  - Efficacy
  - Quality of life
  - Toxicity

Safety in Clinical Trials

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- Clinical Trial Protocols
- Informed Consent
- Institutional Review Board (IRB), ethics committee or Human Research Ethics Committee (HREC)
- Data Safety Monitoring Board (DSMB) or Data Monitoring Committee (DMC)

Clinical Trial Myths

Researchers treat patients like guinea pigs.

Clinical trials are only appropriate for people who have exhausted all of their standard treatment options.

I won't have access to the experimental treatment once a clinical trial ends, even if it's working.

Clinical Trial Facts

Fact: Patients and caregivers are the heart and soul of clinical trials.

Fact: Clinical trials provide access to promising new treatments that may be more effective than the standard approach.

Fact: Patients can continue on the experimental treatment, even if the trial ends, as long as it is working, or until the experimental treatment is approved and becomes available.

Clinical Trial Myths

Patients may receive a placebo instead of treatment.

Clinical trials cost more than regular treatment.

Clinical Trial Facts

Fact: Patients will always receive either the new treatment or best-known current treatment. Sometimes a placebo is given in addition to the best-known treatment as a way of blinding the trial.

Fact: The trial sponsor or insurance should cover medical costs.

Some of the Benefits of a Clinical Trials

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- Clinical trial participants receive close monitoring care and support by a research team who understands their disease.
- Access to new drugs and treatments that may be more effective, before they’re widely available.
- You can take a more active role in your own health care.
- Making an important contribution to research, that may help other people who have the same illness.
- Being among the first to benefit, if the drug or treatment is found to be helpful.
- You can back out of a clinical trial at any time.

Finding Clinical Trials

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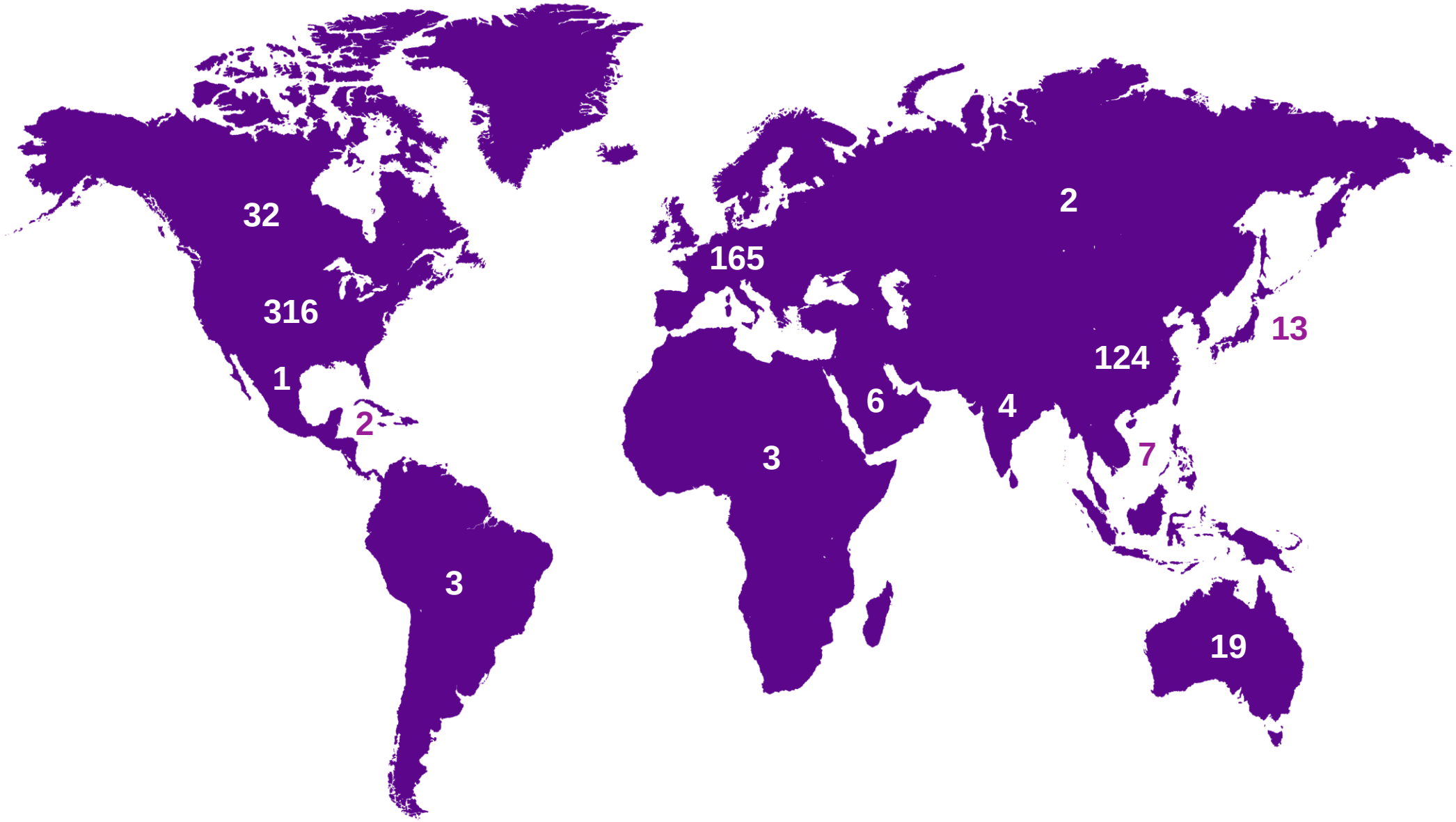
- **Healthcare Providers**
- **Support Groups**
- **Industry Websites**
- **Online Clinical Trial Finders**
  - Clinicaltrials.pancan.org
  - Trials.cancer.gov
  - Ecpc.org/edu/clinical-trials-database
  - Clinicaltrials.gov
  - Australianclinicaltrials.gov.au/anzctr_feed/form

Open and Enrolling Pancreatic Cancer Clinical Trials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SCCC Clinical Trial

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A phase I/II study of safety and efficacy of Ribociclib (LEE011) in combination with Trametinib (TMT212) in patients with metastatic or advanced solid tumors

- Open-label
- Non randomized
- Dose escalation of two FDA approved anticancer drugs
- Combination of a MEK inhibitor and a CDK4/6 inhibitor

Design and Objectives

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Phase Ib:

- To define the maximum tolerated dose (MTD) of ribociclib and trametinib

Phase II:

- To assess overall response rate (ORR) with the drug combination in patients with:
  - Advanced or metastatic pancreatic adenocarcinoma who have failed at least one prior systemic treatment (Arm 1)
  - Advanced or metastatic KRAS-mutant CRC who have failed at least two prior lines of treatment (Arm 2)

Secondary Objectives

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- To characterize the pharmacokinetics (PK) of the drug combination.
- To assess the preliminary anti-tumor activity.
- To evaluate:
  - Safety and tolerability of drug combination
  - Duration of response (DOR)
  - Disease control rate (DCR)
  - Time to response (TTR)
  - Overall survival (OS)
  - Progression-free survival (PFS)

Eligibility

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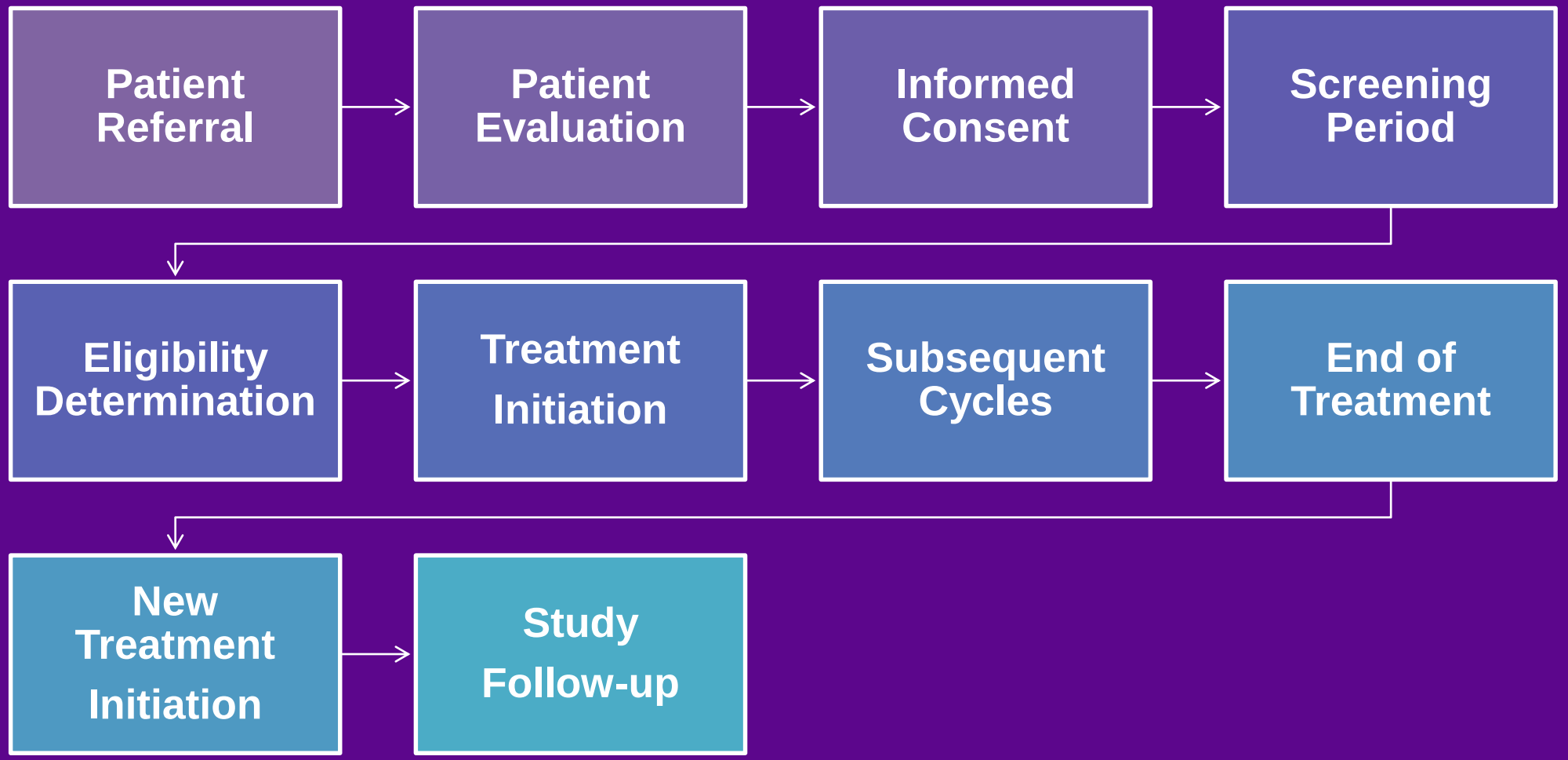
- Able to give written informed consent.
- Advanced malignancy that has progressed or recurred following standard therapy.
- Patient must have measurable disease.
- An estimated life expectancy of at least 3 months.
- Adequate organ function as indicated by laboratory values.
- Women of childbearing potential must have a negative serum pregnancy test.
- Willing to use an effective means of contraception in addition to barrier methods.
- Excellent performance status.

Clinical Perspective on Clinical Trials

Clinical Trial Process at Sylvester Comprehensive Cancer Center



Patient Referrals at Sylvester Comprehensive Cancer Center

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External Referrals

- Clinical trial websites
- Study sponsors
- Next generations sequencing
- Other institutions

Internal Referrals

- Internal physicians
- Other oncologist
- Surgeons
- Primary care physicians

Patient coordinator/nurse navigator contacts the patient for registration and medical record collection.

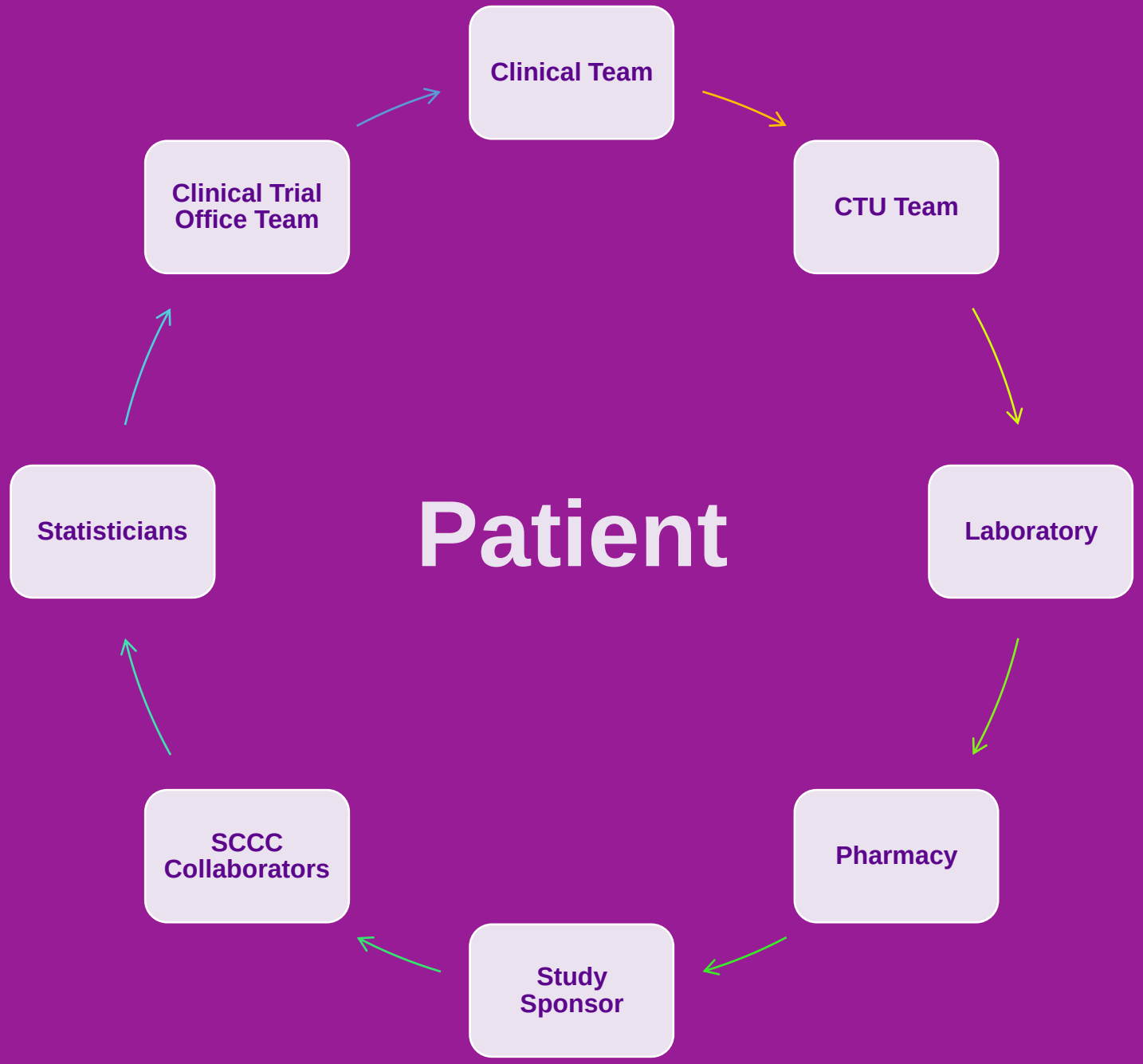
Medical records are reviewed by clinical study team and patient is contacted for phone interview, if deemed eligible.

Patient is scheduled for evaluation by our clinical trial's investigators.

Patient Evaluation

- Patient is evaluated and medical records are reviewed by our pancreatic oncologist.
- Previous and optional treatment regimens, including clinical trials are reviewed with patients.
- If patient’s best treatment option at the time of evaluation is a clinical trial and patient meets eligibility, clinical trial consent is provided to patient.
- Patient is then seen in follow up visit to review consent (ICF) and proceed with screening procedures.

Multidisciplinary Approach of Clinical Trials at SCCC



What to expect during your clinical trial evaluation

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Informed Consent

- Is this the right clinical trial for you?
- Reason for the trial.
- Risk/benefits associated with participation.
- Description of all procedures requirements.
- Consent is signed in clinic.
- Patient is free to withdraw from study at any time.

Screening Period & Eligibility Determination

- Approximately 10-14 days.
- Medical procedures are performed such as labs, scans, EKGs, etc.
- All medical procedure results are reviewed.
- Patient is informed of eligibility and date of clinical trial initiation.

Treatment Initiation

- Administration of first dose of study medication.
- Frequent clinic visits for exams, labs and to review side effects.
- Treatment of any side effect.
- In subsequent cycles visit become less frequent.
- Scans are obtained to determine response to study treatment.

End of Treatment & Study Follow-Up

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- Patients may continue on study treatment until:
  - Disease progression
  - Occurrence of unacceptable toxicity
  - Withdrawal of consent by patient
  - Patient is lost to follow-up
  - Sponsor terminates the study

End of Treatment & Study Follow-Up

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- After discontinuation of study treatment, all patients will be followed for safety except:
  - In case of death
  - Loss to follow-up
  - Withdrawal of consent

- Patients are followed for survival once they discontinue study treatment and efficacy assessments.

- However, if there is evidence of clinical benefits patient may continued to receive study treatment even study has ended.

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