The Benefits of Clinical Research:
Bringing Tomorrow's Drugs to Patients Today

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What is a Clinical Trial?

- A carefully designed study testing safety and effectiveness of a medical strategy, treatment, or device
- Participants must be aware that the research may not benefit them.
- Strict oversight protects participants

What is a Clinical Trial?

- The sponsor and other groups are responsible to oversee the conduct of a trial
  - Sponsors can include the National Institutes of Health (NIH), organizations (PSC Partners), a single physician/university (UT San Antonio) or a pharmaceutical/device company
- Oversight: Ethics Committees, Data Safety Monitoring Board
- Studies follow strict standards to protect patients and are designed to produce reliable study results
- Require written patient informed consent
- Important to understand what “phase” the clinical trial is at

Phase 1: Safety and Dosing

- First time drug is given to humans
- First done in “healthy volunteers” and then in patients with the disease
- Usually only given for 1-14 days
- Not able to show if drug “works” but to show major safety signal
- Small number of patients included
- Typically involves more blood draws and physical exams than normal medical care

Phase 2: Early Effectiveness

- Studies patients with the disease
- Treatment is given for a longer period than Phase 1 to test whether there is a positive response
- Evaluates longer term safety
- Typically, very specific patient population (many exclusion criteria) and may be more difficult to qualify
- Typically, different doses are tested
- Treatment is usually tested against a control
  - Active control: Another drug known to have benefit in patients with the disease
  - Placebo control: A sham pill/injection
- Most times patients will be ”randomized” to receive new treatment or the control (placebo or active)

Phase 3: Large Studies Leading to Approval

- Trials which are the focus of new drug approvals by FDA
- Larger number of patients to test how well the treatment works and to measure side effects
- Some trials compare a new drug to an already approved drug to prove similar/better (active control trials)

Phase 4: Real World Monitoring

- Trials that test approved drugs (e.g., vancomycin vs ursodiol) or procedures (e.g., ERCP stenting vs balloon) in broader patient populations (less exclusion criteria)
- Pediatric trials are usually performed after safety and efficacy are established in adults
- Trials are typically conducted at a larger number of research centers
- Many times, expanded to include more community/regional medical centers

Ongoing Research in Liver Disease

MASH: A New Therapeutic Era

- Metabolic dysfunction associated steatohepatitis affects millions
- Until recently there were no effective medications
- Clinical research is rapidly transforming treatment

Innovative MASH Therapies

- Incretin (GLP-1, Glucagon, GIP agonist) based drugs reduce weight and liver fat
- FGF21 analogues improve metabolic pathways
- RNA therapies target genetic drivers such as PNPLA3

Primary Biliary Cholangitis (PBC)

- Autoimmune destruction of bile ducts
- For decades treatment options were limited
- Clinical trials are expanding therapy dramatically

Next Generation PBC Treatments

- PPAR agonists regulate bile acid metabolism
- Promising results improving liver enzymes and symptoms
- Examples include seladelpar and elafibranor, both currently available in Phase 3 and Phase 4 clinical trials.

Wilson Disease: Toward a Cure

- Genetic disorder causing copper accumulation
- Current therapy controls copper but does not cure
- Gene editing technology currently in Phase 2/3 development aims to correct the underlying mutation and cure disease

Why Participation in Clinical Trial Matters

- Patients gain early access to innovative therapies
- Research accelerates discovery of cures
- Today’s participants make tomorrow’s treatments possible

Clinical Trials at American Research Corporation (ARC)

Who We Are

American Research Corporation (ARC) is one of the largest independent clinical research facilities globally. Based at the Texas Liver Institute in San Antonio, ARC sets the standard for innovation, compliance, and patient care in liver disease research.

1,100+
Peer-Reviewed Publications

50,000+
Liver Visits Annually

2,000+
Cirrhosis Patients Managed

24-Bed
Inpatient Phase I Unit

Premier liver research center | Internationally recognized for contributions in viral hepatitis, MASH, obesity, HCV, HBV, PSC, PBC, and cirrhosis. Published in the New England Journal of Medicine and The Lancet.

ARC Therapeutic Areas & Trial Capabilities

Therapeutic Focus

MASH / NAFLD
2,000-patient phenotyped database

Viral Hepatitis
HCV, HBV – pioneering global studies

Cirrhosis & Portal HTN
2,000+ cirrhosis patients managed

Obesity & Metabolic
Hypertriglyceridemia, Type 2 Diabetes

Autoimmune Liver Disease
PSC, PBC, AIH

Liver Cancer (HCC)
End-stage liver disease and transplant

Clinical Trial Phases

Phase I – Inpatient

- 24-bed state-of-the-art inpatient unit
- Healthy volunteers and patient populations
- All staff CPR/ACLS certified
- 24/7 centralized nursing station

Phase II–III – Outpatient

- Full-service outpatient research unit
- On-site lab, pharmacy, biopsy, imaging
- Secure coded-access limited areas
- Inpatient and outpatient settings

How Can You Refer Individuals for Research?

Simply follow one of these links to submit basic information for

Healthy volunteer opportunities

(you or your patients can submit):

https://txliver.com/research-referrals/

Provider referrals for clinical research trials

(your office can submit):

https://txliver.com/physician-research-referrals/

Final Thought

- Clinical research is how medicine moves forward
- Breakthrough therapies exist today because patients participated in trials
- The next generation of cures is already being tested