

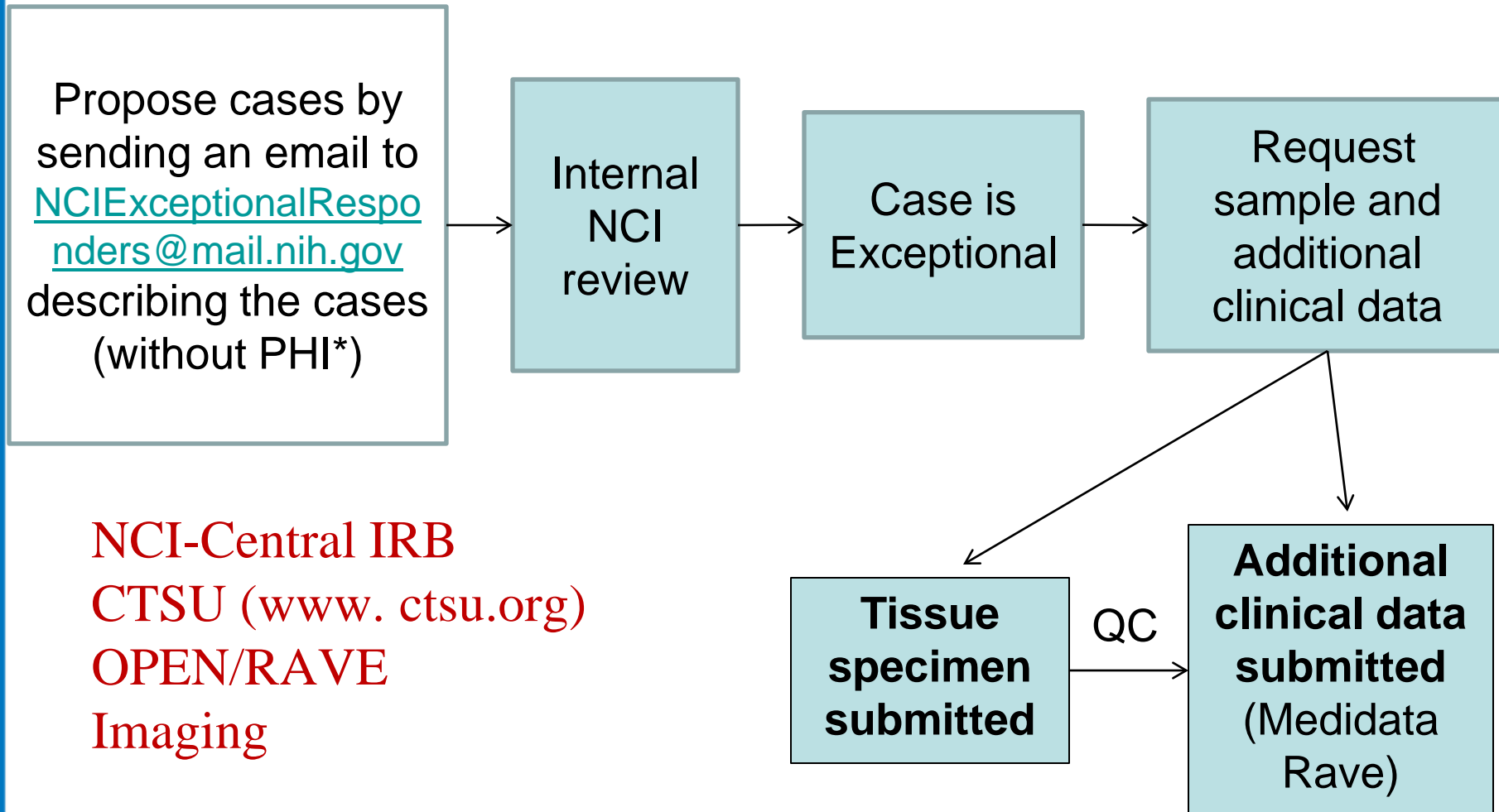
NCI Exceptional Responders Initiative

Protocol 9671
NCT 02243592

Exceptional Responders: Definition

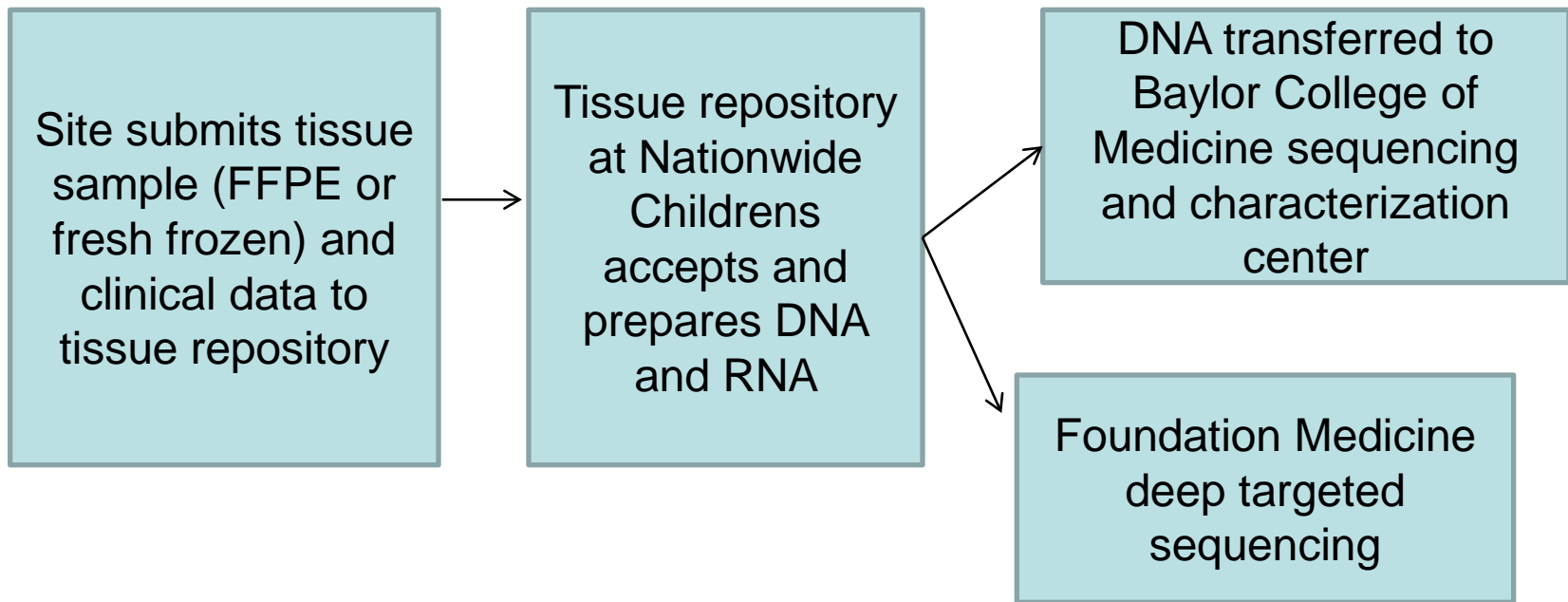
- **Definition of “Exceptional Responder”:**
 - Complete Response, or
 - Partial Response lasting at least 6 months
 - Drug did not go on to FDA approval in that indication due to insufficient activity or
 - Standard treatment not expected to have CR or PR \geq 6 months in $>$ 10% of patients
 - Standard treatment with response lasting 3X longer than the median response duration seen in clinical trials

Exceptional Responder Cases

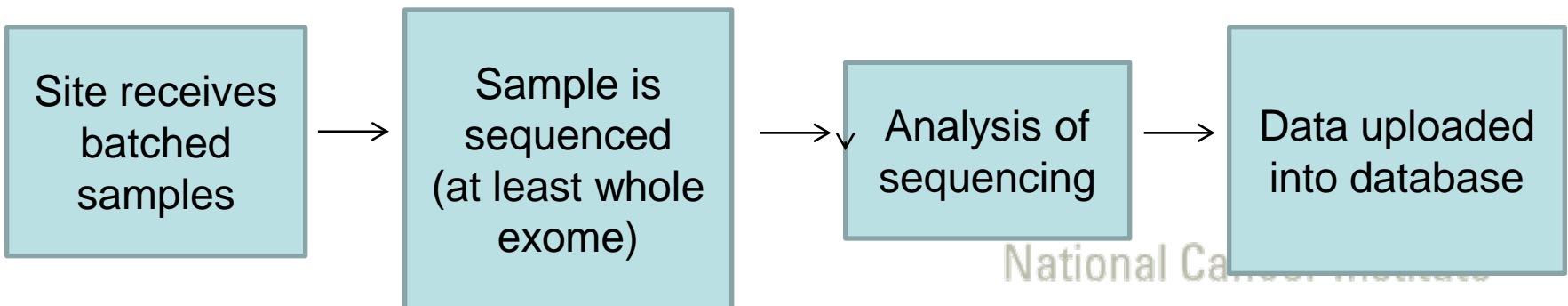


*PHI = protected health information

Submission and Preparation of Samples



Sequencing and Analysis of Samples



Protocol opened September 2014

Cases

- Cases proposed: 138
- Cases conditionally accepted: 72
- Cases on a clinical trial: 29
- Tumors received: 6

Surprises

- Most patients are living
- Most cases are standard treatment

Tumor tissue: Prefer just before drug treatment; otherwise any prior

At least 50% tumor

FFPE, frozen, core acceptable

Normal tissue: blood or other

Cases to date

Tumor type

- Ampulla of vater 1
- Blood: 6
- Brain: 3
- Breast 4
- Colon: 8
- Gastroesophageal: 8
- Head and neck: 4
- Kidney: 2
- Liver: 3
- Lung: 7
- Skin adnexa 1

Tumor type

- Melanoma: 3
- Mesothelioma 1
- Neuroendocrine: 1
- Ovarian: 5
- Pancreas: 6
- Prostate: 2
- Sarcoma 3
- Thyroid: 2
- Uterus: 1
- CUP 1