2026

C3PT TOOL

Platform Trial
Decision Tree

v 1.0

MRCT CENTER
THE MULTI-REGIONAL CLINICAL TRIALS CENTER OF BRIGHAM AND WOMEN'S HOSPITAL AND HARVARD

ITCC
INNOVATIVE THERAPIES FOR CHILDREN AND ADOLESCENTS WITH CANCER

Blood Cancer United

CHILDREN'S ONCOLOGY GROUP

Purpose and Scope of Tool

This tool lays out a stepwise decision framework for determining whether an academic industry collaborative platform trial is the appropriate approach for your trial. It outlines a series of scenario-based questions to assess factors such as multiple therapies, rare populations, shared control groups, feasibility across sites, regulatory capacity, stakeholder acceptance, industry engagement patient advocacy and infrastructure needs. It provides guidance on when these types of platform trials are not suitable and alternative trial designs like single-arm or traditional randomized trials would be more appropriate for your research question(s).

Introduction

The purpose of a **platform trial in pediatric oncology** is to create a flexible, efficient, and adaptive framework for testing multiple therapies within a single overarching protocol. This design is particularly valuable because:

1. **Addresses small patient populations:** Pediatric cancers are often rare, making traditional trials slow and inefficient. A platform trial allows multiple treatments to be studied simultaneously under one structure.
2. **Facilitates rapid adaptation:** New drugs can enter or leave the trial over time without starting a new study, which accelerates evaluation and reduces delays in bringing effective therapies to children and may provide a framework for decision making for patients and families.
3. **Uses shared control groups:** This minimizes the number of patients assigned to control arms, reducing burden and improving ethical acceptability in vulnerable populations.
4. **Supports precision medicine:** It can incorporate biomarker-driven sub-studies, matching therapies to specific genetic or molecular profiles common in pediatric oncology, when appropriate.

The acceptability and functionality of a multi-arm platform trial will vary depending on its design and the stakeholder involved.

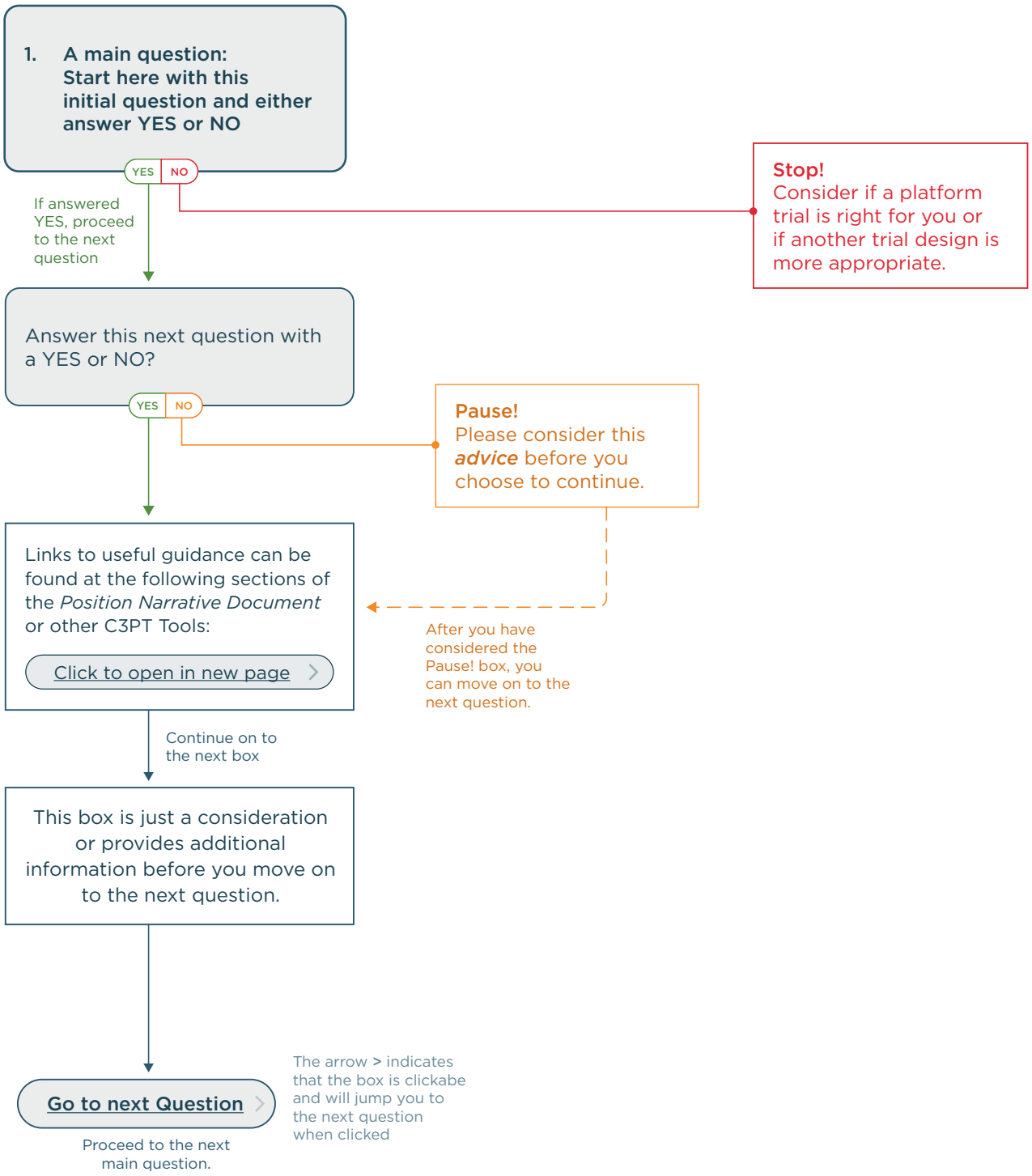
- **For patients/caregivers**, having a multi-arm platform trial could have two major purposes
  - reduced uncertainty of selecting a treatment path when there isn't a generally accepted road map
  - reduced time between treatments, if change is required
- **For industry**, having a multi-arm platform might be appealing to
  - Access to rare, geographically dispersed pediatric population
  - larger companies that need to efficiently fulfill regulatory requirements
- **For regulators**, having a multi-arm platform trial might
  - simplify the communication process for both efficiency and consistency
- **For academia**
  - Better use of resources (for instance, shared controls)
  - Flexibility to be responsive to incoming data and emerging science
  - Economic efficiencies
- **Overall**
  - Shared infrastructure

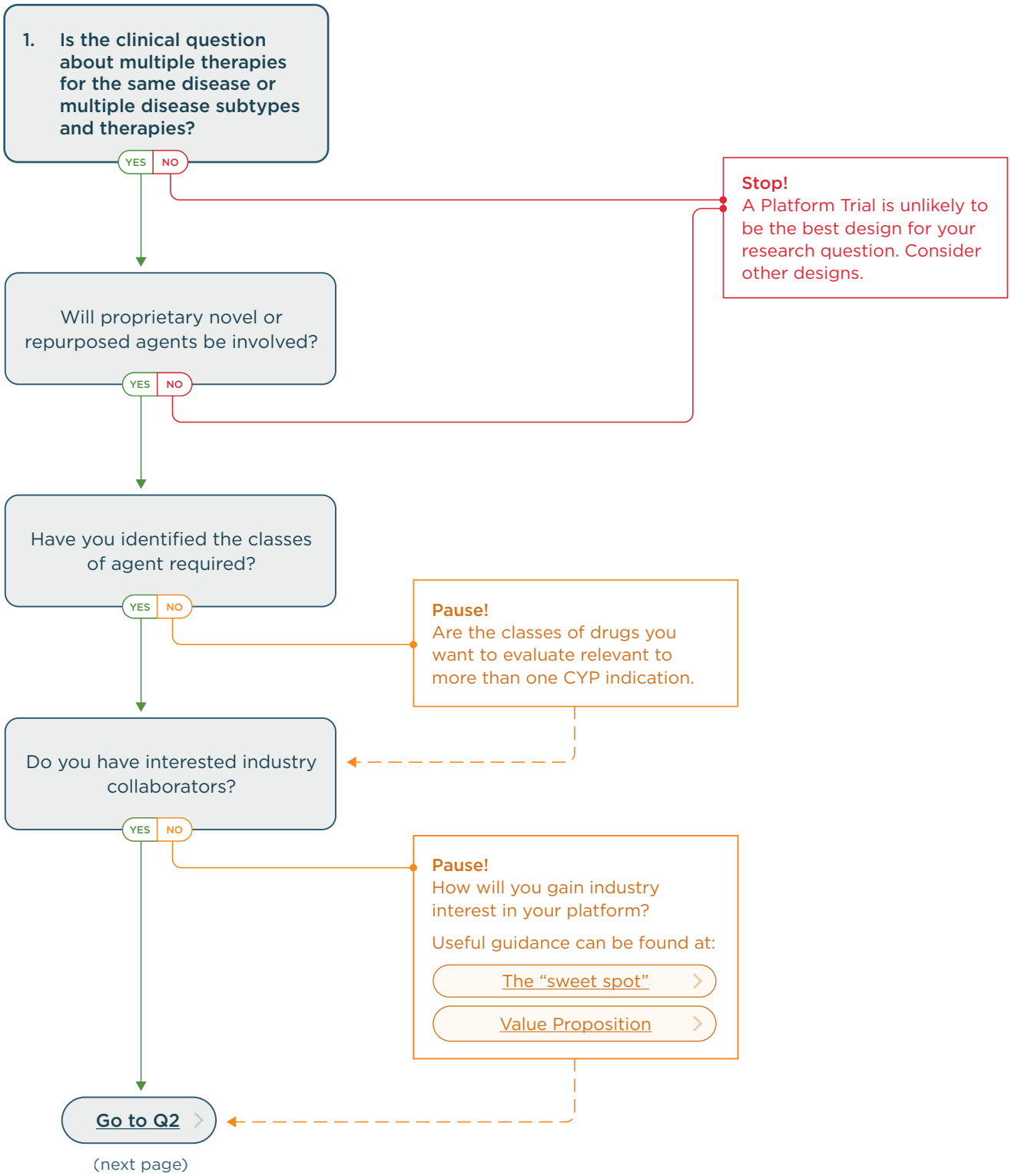
Scenarios when platform trial may be less desirable:

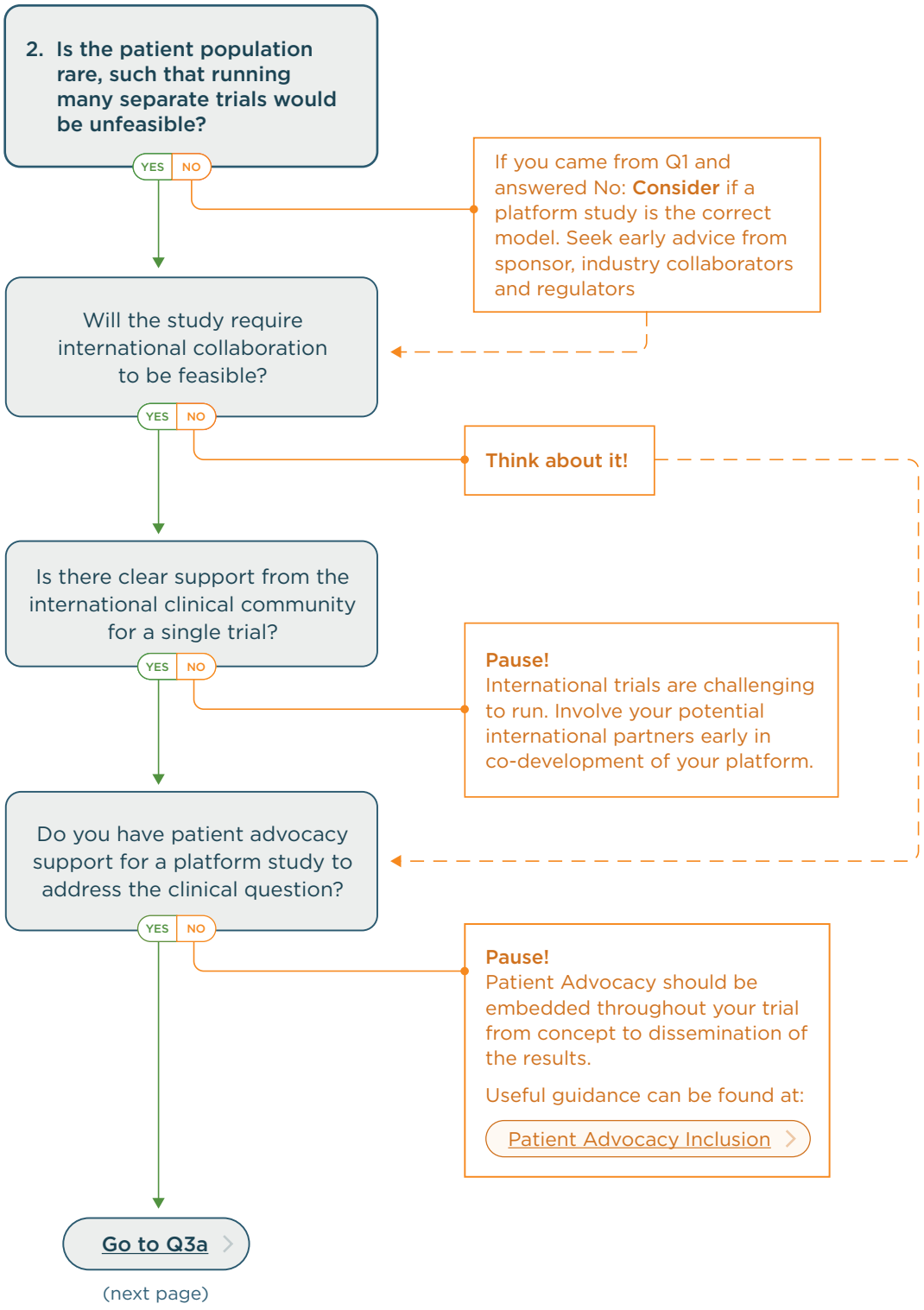
- **Single-agent, single hypothesis** with clear primary endpoint → classic randomized or single-arm trial with historical control.
- **Urgent need for rapid approval of one drug** and there aren't other candidate agents → focused pivotal trial may be faster.
- **Limited infrastructure or funding** for long-term operations → choose smaller adaptive trials or single-arm study design

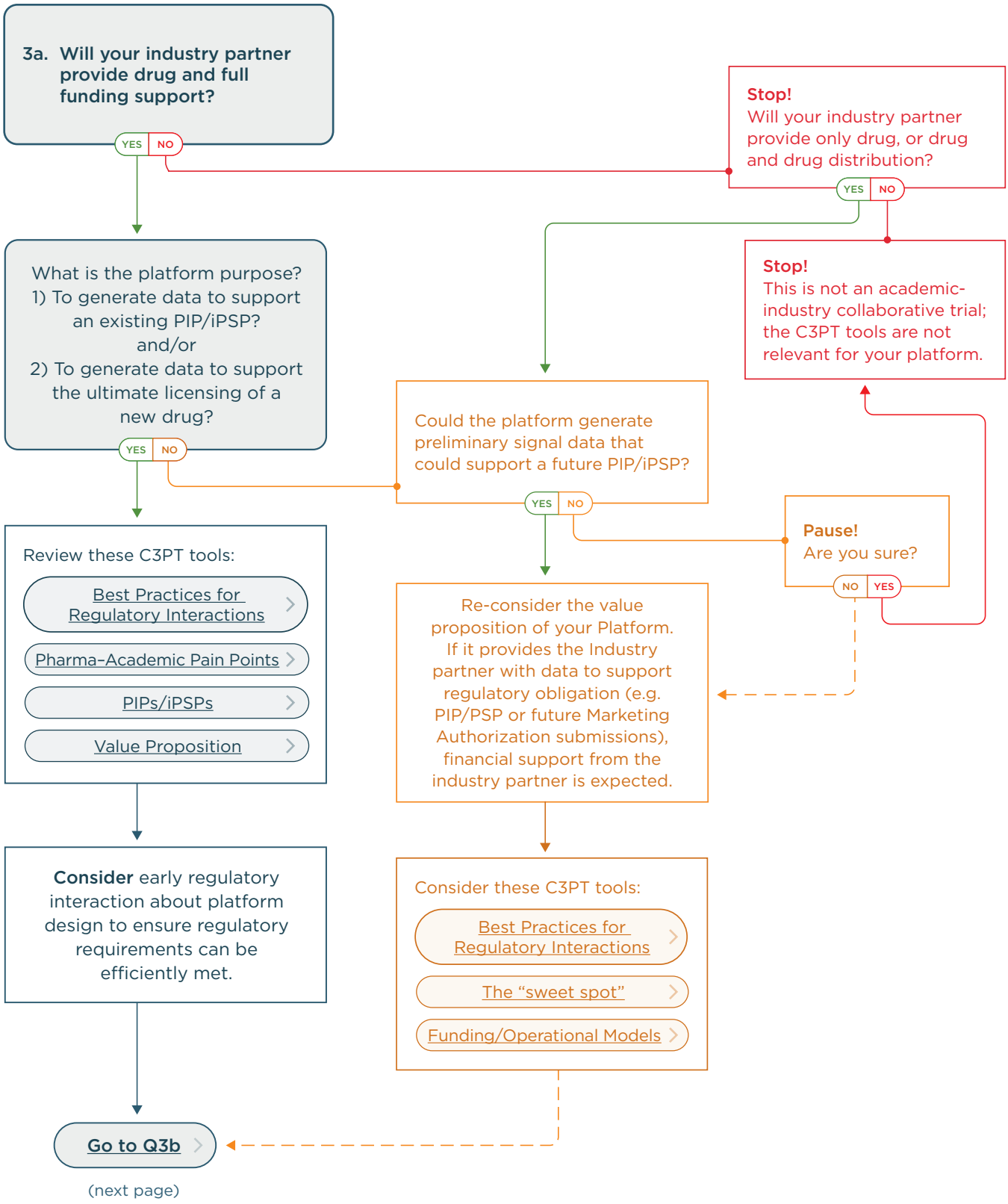
How to Use the Decision Tree Tool

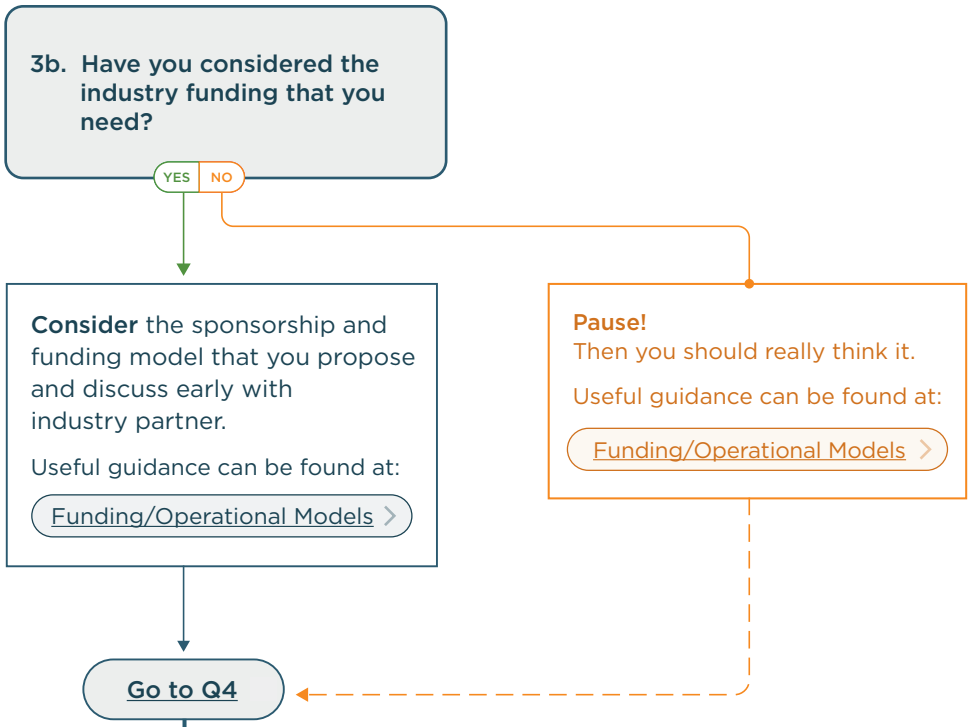
The tree featured on this page is for example purposes only and does not have any functionality. Please review this to understand how the real tree (starting on the next page) works.

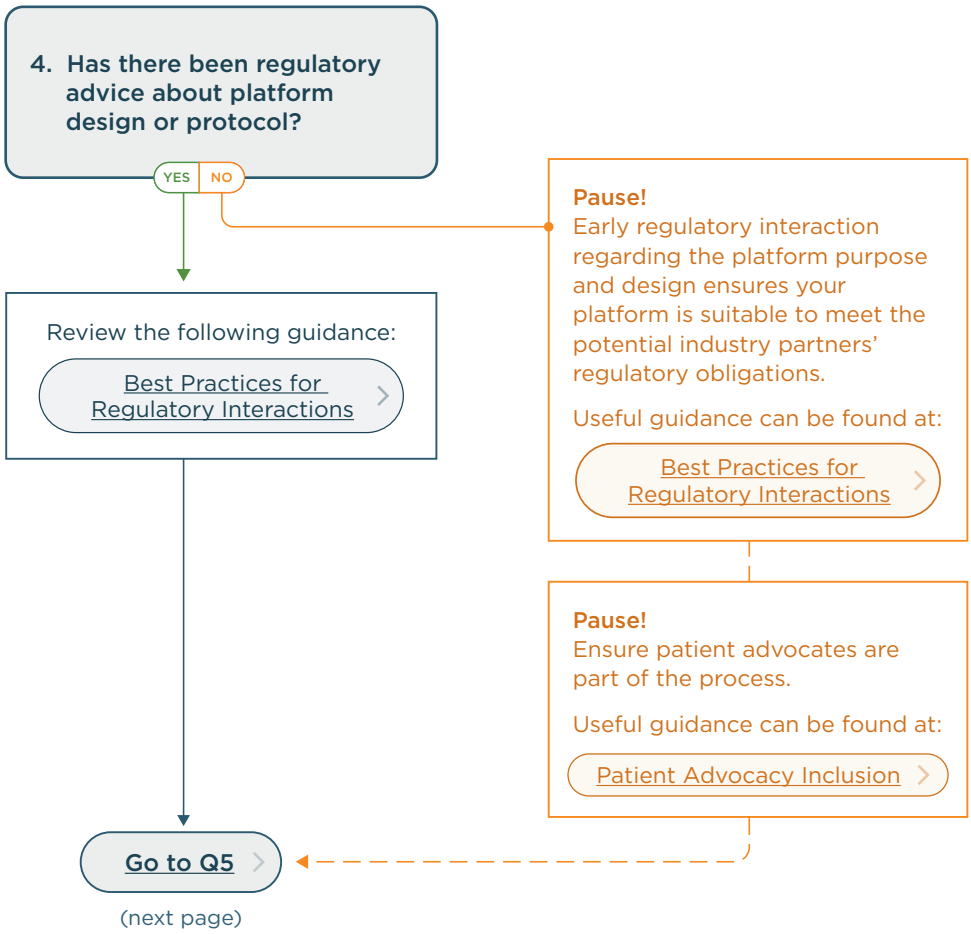












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