

Investigator Initiated Trials Management



Research Overview: Objectives & Methodology

With a significant increase in the number of investigator initiated trial (IIT) submissions occurring in the healthcare industry, it is critical for bio-pharmaceutical companies to properly vet each proposal and approve the ones that demonstrate scientific merit and align with shifting corporate objectives.

Research Overview

- This benchmarking study is designed to provide a detailed roadmap for improving IIT management at bio-pharmaceutical companies, capturing critical metrics and insights on IIT management team structure, staffing, budget and performance metrics.

Research Methodology

- Best Practices, LLC engaged 30 leaders from 23 bio-pharmaceutical and medical device companies through a benchmarking survey.
- In-depth interviews were conducted with 4 executives to gather more detailed insights related to investigator initiated trials or studies.

Topics Covered:

- IIT Submission Review and Systems
- Functional Involvement
- IIT Funding
- IIT Audit
- Future Improvements
- Challenges
- Important Criteria for IIT Evaluation
- Key Reasons for Rejecting IIT Proposals

Universe of Learning: 23 Companies Participated in Study

This study engaged 30 executives representing 23 leading bio-pharmaceutical, medical device and other healthcare companies. Nearly 60% of participants serve at the level of director or above.

Benchmark Class:

Boehringer
Ingelheim

FUJIREBIO[®]
Diagnostics, Inc.

GALDERMA

pharmacyclics[®]
An AbbVie Company

Santen

COOK[®]
MEDICAL

Alnylam[®]
PHARMACEUTICALS

smith&nephew

Allergan

KYOWA KIRIN

BAYER

abbvie

Shire

ucb
Pharma

MERCK

Pfizer

HORIZON
P H A R M A

dexcom[®]

Vifor Pharma

FRESENIUS
MEDICAL CARE

GRIFOLS

Medtronic

SANOFI

Key Findings and Insights

few of the key findings and insights drawn from this study:

Performance

- **“Number of Publications” is Top-Rated IIT Performance Metric:** The top-rated IIT performance metric is total number of publications resulting from a study, a metric which 53% find highly effective. Close to one-third of companies also find the time taken by the review committee to approve a proposal and the total number of submissions received/approved to be highly effective ways of measuring overall IIT performance.
- **Strategic Fit and Scientific Merit are Top Determinants of IIT Approval:** The most highly rated criteria for evaluating new IIT proposals include “fit into company strategy” and “scientific merit.” Other important factors used by companies to evaluate IIT proposals include “reasonable budget,” “investigator qualifications,” and “study feasibility.” Conversely, the top reasons for which companies reject IIT submissions are strategic misalignment and high costs.
- **A Central Digital Management System is Key to IIT Efficiency:** The use of a centralized, standardized digital management system is a key component of an efficient IIT review process. Forty-one percent of companies also cite the importance of a streamlined review and approval process, while 37% cite regular pre-defined meetings as critical to an efficient IIT review process.
- **Quality and Budget are Top Parameters for IIT Auditing:** The top parameters for auditing IITs include quality (which 87% of companies use), budget (78%) and governance (70%).

Medical Affairs Leads All IIT Activities, but Thrives with Support from Many Other Functions

Medical Affairs drives most work in investigator initiated trial programs. Still, other groups contribute to overall program success. When two bubbles appear in a cell, it means the benchmark class was split on its role, with no clear role attached.

IIT Leadership Roles:

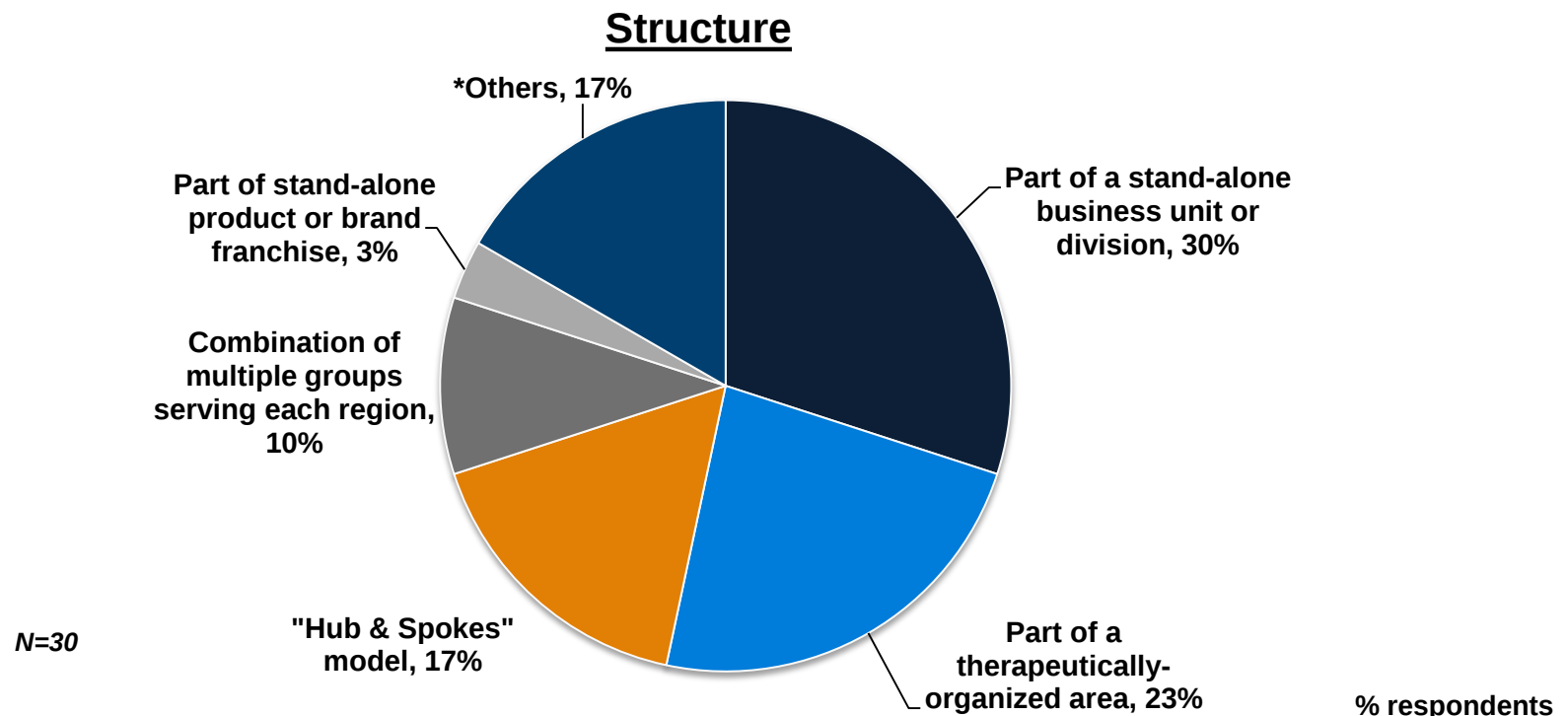
	IIT Process	IIT Oversight	IIT Data Use	IIT Publication
Medical Affairs	●	●	●	● / ○
Regulatory	○	○ / ○	○	○
R&D	○	○ / ○	○	○
Publication	○ / ○	○	○	○ / ●
Marketing	○	○	○ / ○	○
Commercial	○	○	○	○
Market Research	○	○	○	○

- Lead/Co-lead
- Support
- No Involvement

IIT Management Structure Should be Tailored to Fit Organization: Most Use Stand-alone Business Unit or Therapy-Oriented Model

Throughout the industry, companies use many different structures to effectively manage their IIT programs. Thirty percent use a stand-alone group that is part of a business unit or division, with another 23% using a similar therapeutic area-aligned model. Several other firms use a hybrid model that may entail a combination of teams serving different regions or operational levels.

6) How is your IIT management structured?

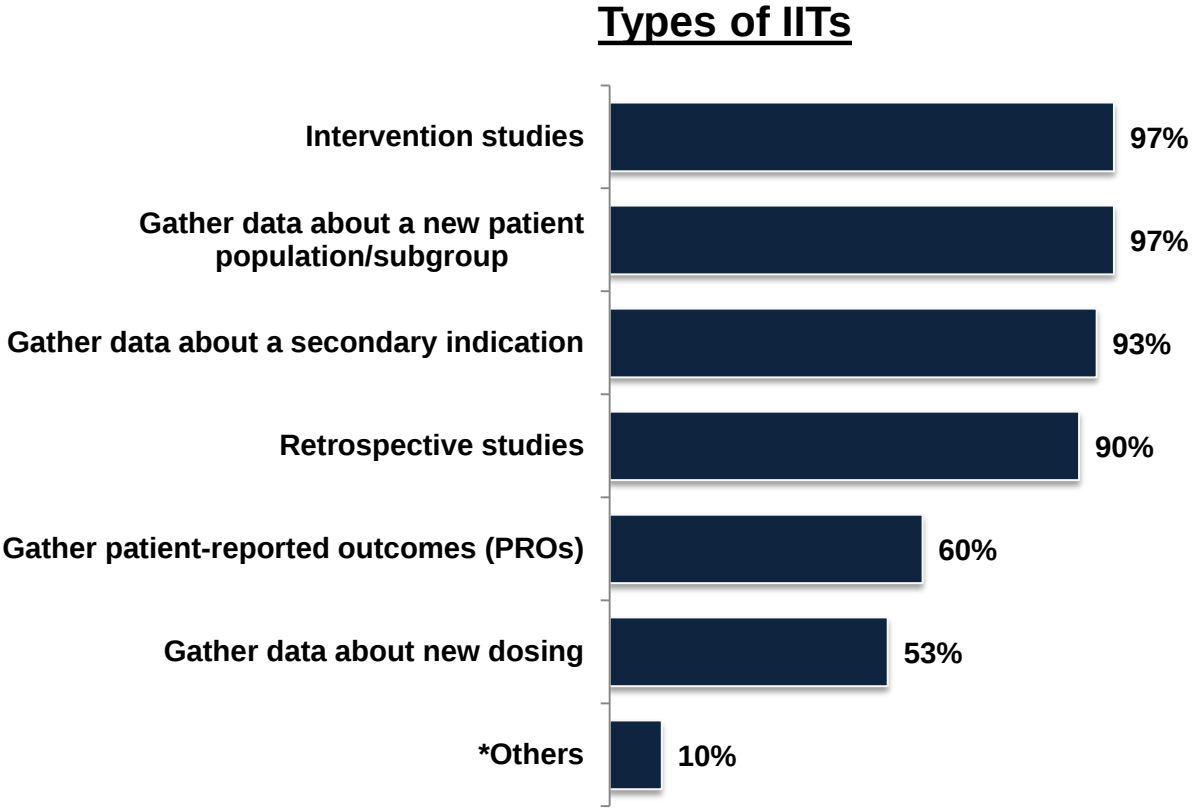


***Other IIT Management Structures Used:** Central Operations support, Part of Medical Affairs, Centered through Medical Affairs with involvement from other R&D functions and linked to the Commercial Business Units, OTC, Rx, and A&C IITs are managed by an IIT Coordinator within Clinical Development, headquartered operations and management for global program

Most Companies Support a Multitude of IIT Study Types

Nearly every company (97%) supports both intervention studies and studies which gather data about new patient populations. In addition, 93% support secondary-indication studies, while 90% support retrospective studies. Studies probing patient-reported outcomes and dosing are less common overall, but still supported by the majority of surveyed organizations.

7) What type of IIT studies does your organization support?



% respondents
N=30

***Others:** Observational, Animal, In Vitro, In Vivo, Patient observation and monitoring of blood parameters

“If the gap is something that could be addressed with a study that’s looking at some sort of retrospective data we would be definitely open to that.”

--- Associate Director, Global Medical Affairs

“In patient-reported outcomes, data from the doctor is important. We lack our own internal expertise or the competency for such studies.”

--- Head, Medical Affairs, U.S. Operations

Access to Resources and Delays in Approval and Reviews are Most-Frequently Cited IIT Hurdles

Approximately one-third of all participants cited “access to management/resources” and “delays in approval, review and contracting” as the top challenges facing companies throughout the IIT management process.

41) What are the top challenges to the IIT management process?



“The reviews have always been a challenge because of the model of review. We have a centralized committee and the problem with the central committee is that you don’t always have an expert in research or the particular therapeutic area in the review team. So we keep going back and forth for reviews.”

-- Associate Director, Global Medical Affairs

***Other challenges include:** Unhappy investigators, Effectiveness, Prioritization

IIT Committees Average 11 Members, with 42% Enjoying Voting Rights

IIT committees are composed of an average of 11 members across the industry, with roughly 42% of all committee members enjoy voting privileges on IIT evaluation and selection. Voting rights are spread across an average of four different functions represented on the IIT committee.

20) Please estimate the following information with respect to IIT committee membership and voting privileges.

IIT Committee Members & Voting Privileges

	How many IIT committee members are there in total?	How many IIT committee members vote?	Percent of voting members (Normalization Ratio)	Functions with voting privileges on committees that segment voting privileges
Max	30	12	80%	11
75th Percentile	14	8	53%	5
Mean	11	6	42%	4
Median	10	6	37%	4
25th Percentile	6	5	33%	2
Min	3	2	13%	0

“All of our committee members have voting rights. It includes representatives from global and area medical affairs, statisticians, clinical development, discovery in case of preclinical proposal, and regulatory affairs.”

“There are a lot of people that want to be at that table for the review, and of course the more people you have in a review committee more interested they become. So we are constantly struggling about what's the minimum number of reviewers that we really need to conduct the reviews.”

-- Associate Director, Global Medical Affairs

Participant Demographics

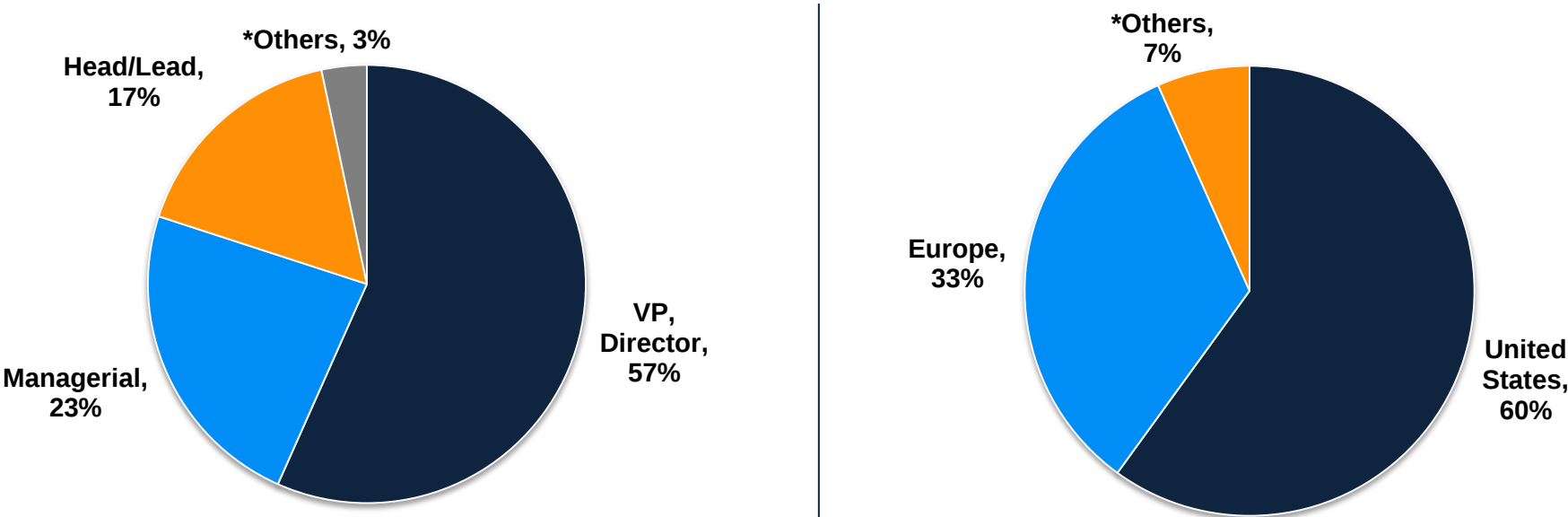


Bulk of Respondents Serve at the VP or Director Level

Nearly 60% of the study participants work at the VP or Director level, with another 17% serving as Heads of units. Sixty percent of the participants work out of the U.S. market.

Q) Please provide the following information, which will be used for classification purposes and to ensure that you receive a copy of the study deliverable.

Participant Demographics



*Others: Senior Product Specialist

N=30

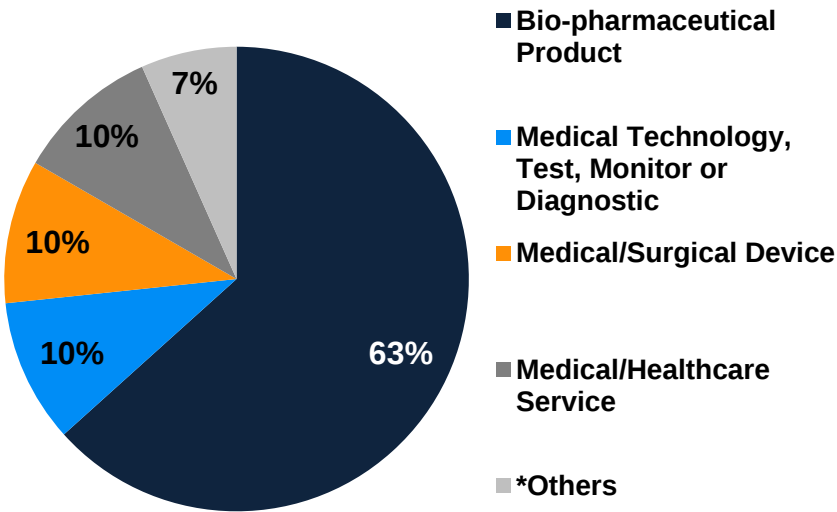
% respondents

Participants Support Bio-Pharma & Other Products around the Globe

Some 63% of participating companies support bio-pharmaceutical products, while the remainder of the benchmark class support other medical technologies, devices or service. About two-thirds of participants have a global IIT management responsibility, while one-quarter support U.S. operations.

2) Please classify your unit's primary product focus.

Primary Focus



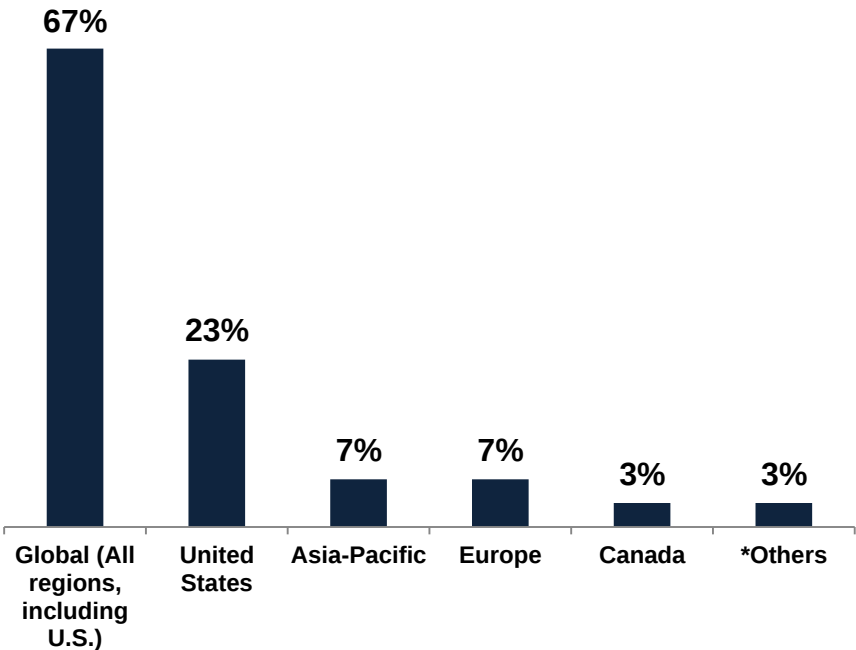
*Others include device, drug, OTC, plasma-derived therapeutic products

N=30

% respondents

3) Please indicate what region(s) you are responding for.

Geographic Responsibility



*Others include Australia and New Zealand

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Best Practices®, LLC is an internationally recognized thought leader in the field of best practice benchmarking®. We are a research, consulting, benchmark database, publishing and advisory firm that conducts work based on the simple yet profound principle that organizations can chart a course to superior economic performance by leveraging the best business practices, operating tactics and winning strategies of world-class companies.

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