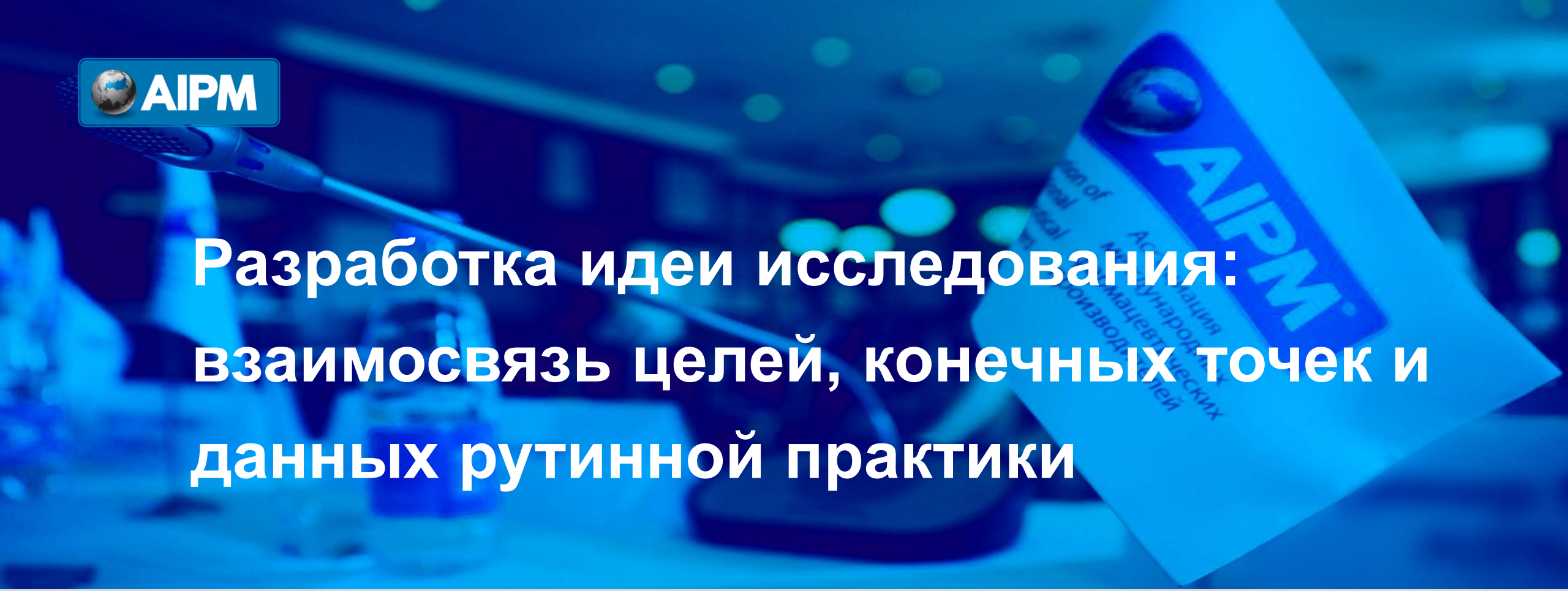


Разработка идеи исследования: взаимосвязь целей, конечных точек и данных рутинной практики

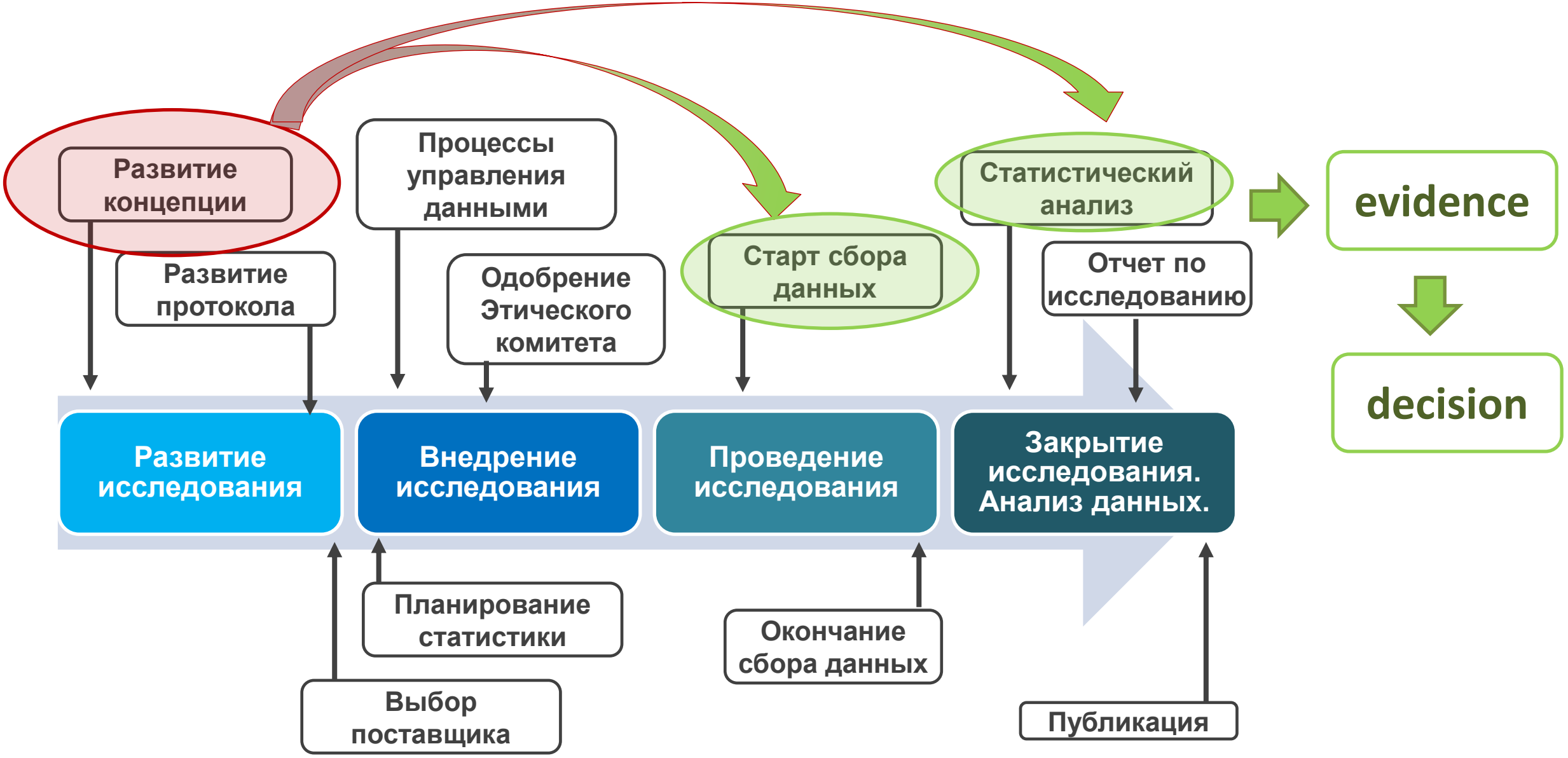
Проект «Диалоги о Real World Data»

Гольдина Татьяна, к.б.н.,
руководитель рабочей группы AIPM по RWE

02 декабря 2022г

Схема разработки и проведения исследования

AIPM Association of International Pharmaceutical Manufacturers Ассоциация международных фармацевтических производителей



Ключевой принцип разработки концепции

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

НЕТ ВМЕШАТЕЛЬСТВА – **ОЦЕНКА РУТИННОЙ КЛИНИЧЕСКОЙ ПРАКТИКИ**

Ключевой вопрос: как … в рутинной клинической практике?

Например:

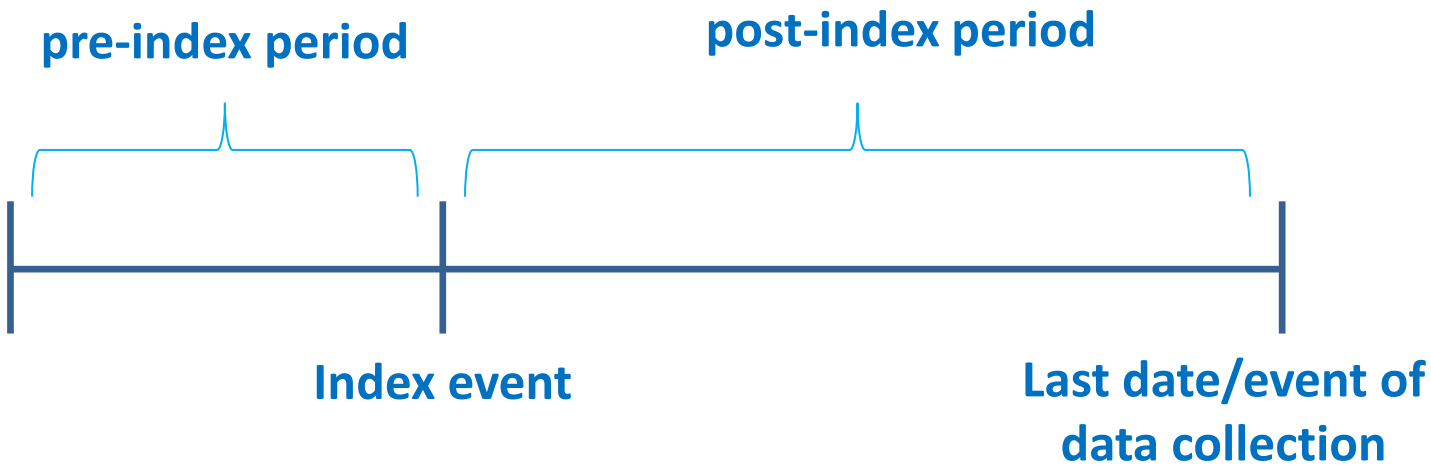
- Что описано в руководстве по лечению заболевания и как это происходит в рутинной клинической практике?
- Какие типы/паттерны лечения я знаю в рутинной клинической практике?
- Какие пациенты в рутинной клинической практике?
- Как часто пациенты в рутинной клинической практике ходят на визиты к врачу?/Как долго в рутинной клинической практике длится госпитализация
- И т.д.

Схема сбора данных/Схема наблюдения

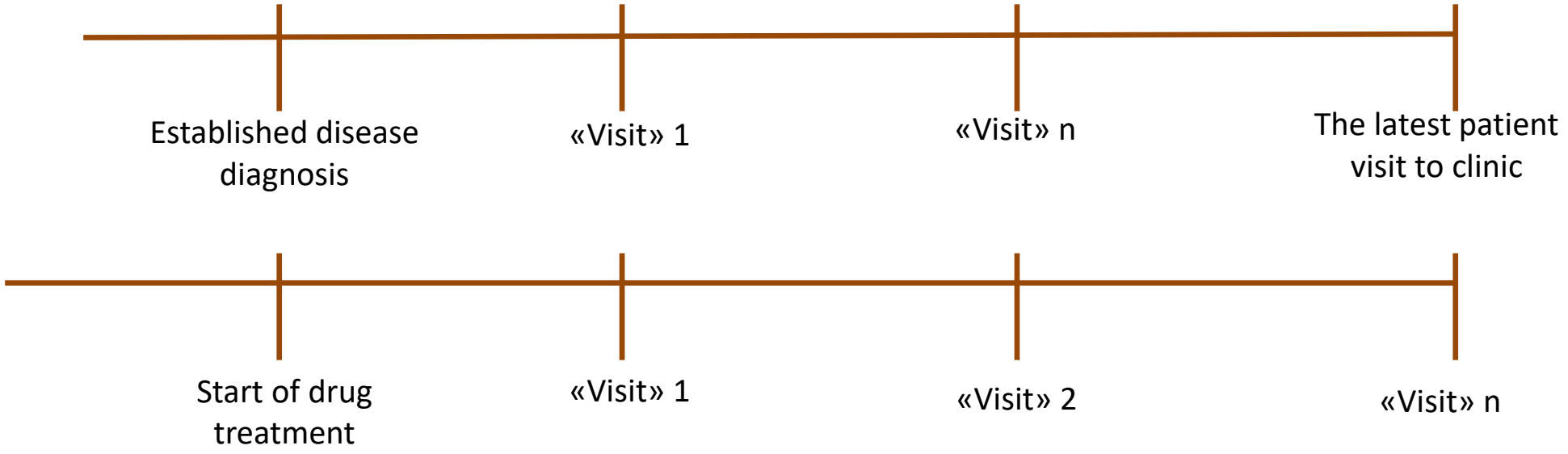
Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

The ***index event*** is defined as the first event in patient disease history which is point of start data collection

Examples:
- The first dose of drug taken (for drug study)
- Basic diagnose/relapse is confirmed by special methods

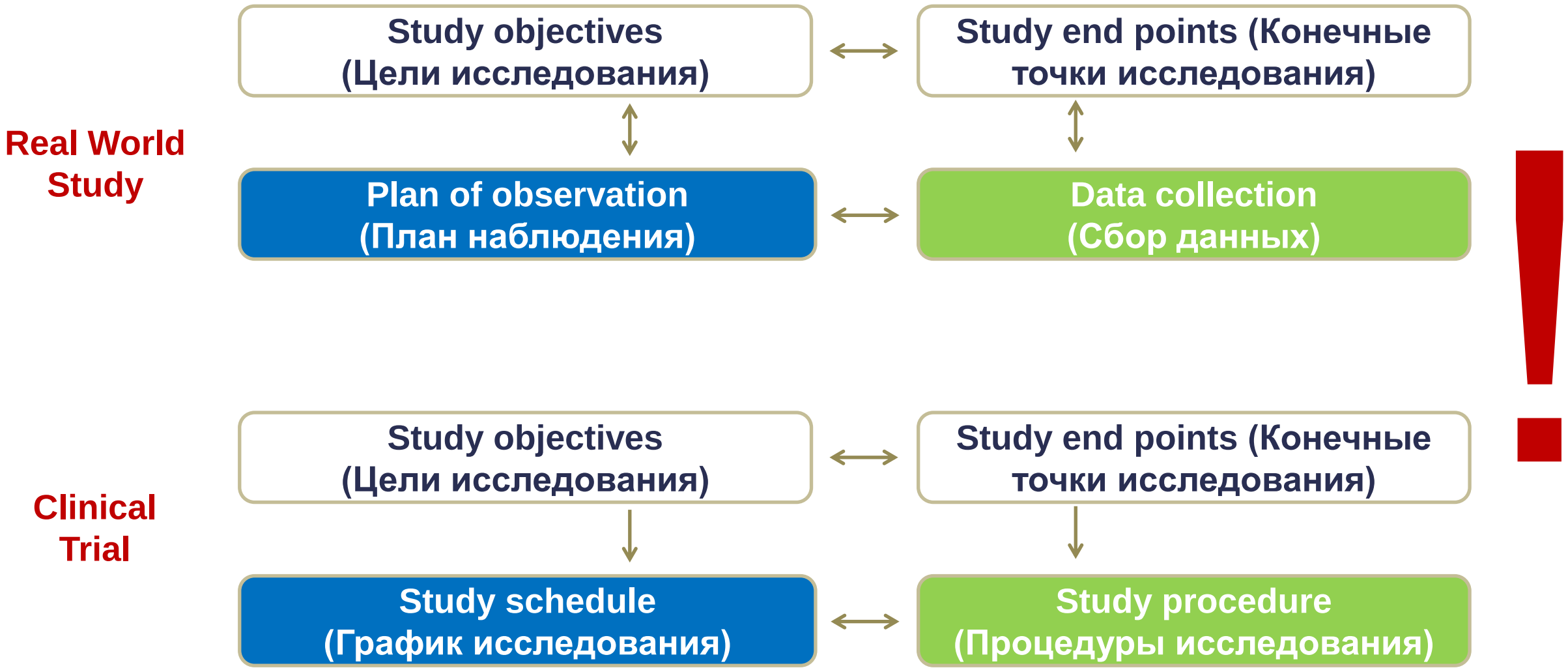


Examples



Планирование исследования

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей



Study objectives and study end points

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

Study objective is a task

Study end point is a measurement of study objective

➢ **Use verb**

E.g.: To describe body weight changes among patients with T2D initiating *DRUG* within 6 and 12 months post-initiation in a real-world setting.

➢ **Use noun**

E.g.: Absolute and relative mean (SD) change in weight from baseline to 3, 6, and 12 months

Study end point should have:

- Exact parameter of measurement,
- Time frame of measurement

Classic rule: 1 objective = 1 end point

In heterogeneous patient population and needs to collect maximum data from routine practice it's difficult to follow classic rule

Often:
1 objective = some end points

Study objectives and study end points

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

Primary (Первичные)

- Always 1 objective – the most priority and jointed all population (despite heterogeneity)

Secondary (Вторичные)

- 5-7 objectives

Exploratory (эксплораторные, или поисковые)

- Not obligatory.
- Included if we need to receive results in new area of researched questions
- Included if we are not sure that sample size will be enough (e.g. due to missing data)

RW study objectives, end points and RWD – useful tool

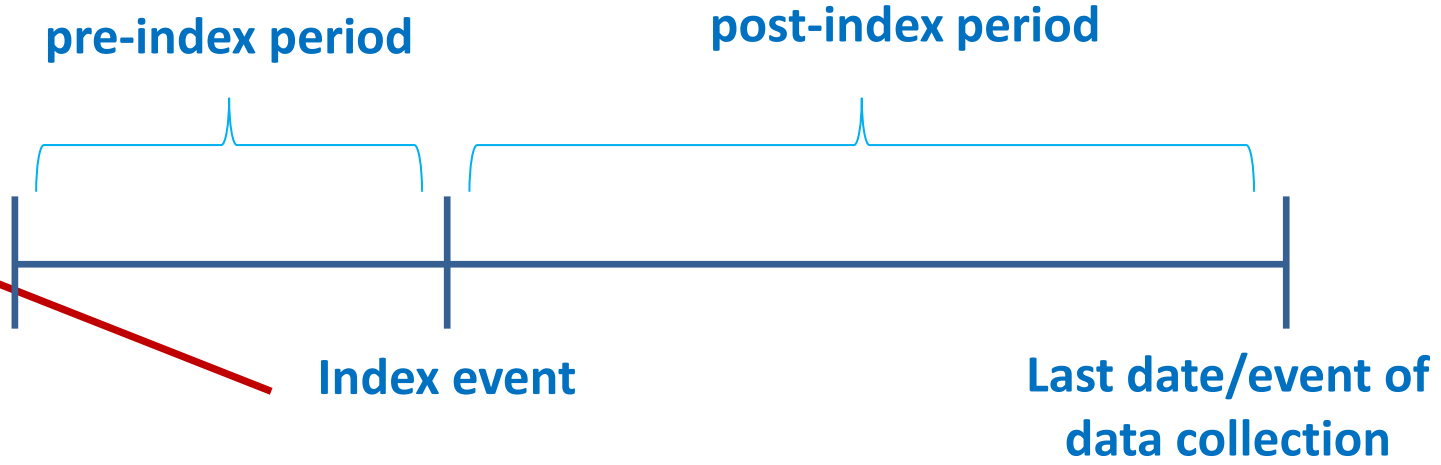
Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

Level	Study Objective	Study End points	Data needed to receive evidence			
			Baseline	Visit/point	Visit/point	Data source
Primary						
Secondary						
Secondary						
Secondary						
Secondary						

Study objectives, end points and RWD – useful tool

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

Data needed to receive evidence			
Baseline	Month 3	Month 6	Data source



RW study objectives, end points and RWD – useful tool

AIPM Association of International Pharmaceutical Manufacturers Ассоциация международных фармацевтических производителей

Level	Study Objective	Study End points	Data needed to receive evidence			
			Baseline	Month 3	Month 6	Data source
	To evaluate HbA1c level dynamic at month 6 in total population	Percentage of patients achieved individual target HbA1c defined by treating physician by Month 6	predefined individualized HbA1c level	N/A	HbA1c	
		Change From Baseline in Glycosylated Hemoglobin (HbA1c) Level at Month 6	HbA1c	N/A	HbA1c	
	To evaluate HbA1c level dynamic over Time at month 6 in total population	Change From Baseline in HbA1c Level Over Time	HbA1c	HbA1c	HbA1c	

The key task is to match data and end points

As a result: listings of required data

Data collection overview

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

Data collection overview is a table which match required data and indexes' periods

Data needed to receive evidence		
Baseline	**Month 3**	**Month 6**
predefined individualized HbA1c level	N/A	HbA1c
predefined individualized HbA1c level	HbA1c	N/A
predefined individualized HbA1c level and HbA1c	HbA1c	HbA1c

Data	Index date	Month 3	Month 6
Targeted HbA1c, FPG and PPG	+		
HbA1c, FPG and PPG	+	+	+

Why is important to develop data collection overview?

Association of International Pharmaceutical Manufacturers
Ассоциация международных фармацевтических производителей

data collection overview

- **Matching of study end points and RWD**
- **Detailed data mapping**
- **Detailed list of variables in the study protocol**
- **Development of CRF for prospective study**
- **Exact data transfer for retrospective study**

Good quality of:

- data,
- evidence

Основные разделы концепции исследования

AIPM Association of International Pharmaceutical Manufacturers Ассоциация международных фармацевтических производителей

Study outline sections	The main description	
Study title	Short (if applicable) and full study title	
Study rationale	RWE gaps. Study design, study population, study objectives	
Study objectives	As minimum – primary, secondary and safety	02.12.2022
Study endpoints	As minimum – primary, secondary and safety	
Study population	Criteria inclusion and exclusion	примеры 23.12.2022
Study design	Prospective/retrospective, comparative (if app). Rationale of the study design.	
Study plan	Plan of visits = plan of patient's observation	23.12.2022
Data collection overview	Main parameters/area of data which are collected in the study by visits	
Study timelines	Start and end of data collection, Study report	
Statistical methods	Population for analysis. Basic methods	16.12.2022
Sample size calculation	Statistical justification of sample size	

Спасибо за внимание!