



RAFAEL

Pharmaceuticals, Inc.

An Affiliate of Rafael Holdings (NYSE:RFL)

"To Save A Life Is To Save A Universe"

Leveraging Relationships in **Ultra-rare Cancers:** *the laser-focused approach*

Sanjeev Luther, President & CEO
August 2021



Safe Harbor Statement

This presentation contains forward-looking statements. These statements relate to future events or the company's future financial performance. In some cases, you can identify forward-looking statements by terminology such as "may", "will", "should", "expect", "plan", "anticipate", "believe", "estimate", "predict", "potential" or "continue", the negative of such terms, or other comparable terminology. These statements are only predictions. Actual events or results may differ materially from those in the forward-looking statements as a result of various important factors. Although we believe that the expectations reflected in the forward-looking statements are reasonable, such statements should not be regarded as a representation by the company, or any other person, that such forward looking statements will be achieved. The business and operations of the company are subject to substantial risks which increase the uncertainty inherent in forward-looking statements. We undertake no duty to update any of the forward-looking statements, whether as a result of new information, future events or otherwise. In light of the foregoing, readers are cautioned not to place undue reliance on such forward-looking statements.

Rafael Pharmaceuticals is Focused on Cancer Metabolism

- ❖ *Rafael Pharmaceuticals is a **clinical-stage oncology company** focused on selectively **targeting cancer metabolic pathways** while simultaneously harnessing the immune system*
- ❖ ***Our Lead Molecule: CPI-613[®] (devimistat)** - in multiple clinical trials for different solid tumors and hematological malignancies; encouraging results both as monotherapy and in combination*

We Have 7 Orphan Drug Designations in U.S. and 2 in EU



1. **Pancreatic Cancer**
2. **Acute Myeloid Leukemia (AML)**
3. **Myelodysplastic Syndrome (MDS)**
4. **Burkitt's Lymphoma**
5. **Peripheral T-Cell Lymphoma (PTCL)**
6. **Soft Tissue Sarcoma**
7. **Biliary Tract Cancer**



1. **Pancreatic Cancer**
2. **Acute Myeloid Leukemia (AML)**
3. **Applied for Burkitt's Lymphoma**

The Difference Between Orphan and Ultra-orphan

Common Diseases



Disease	No. of cases in U.S.
Hypertension	~108 Mn
Diabetes	~34 Mn

Rafael is focusing on these orphan and ultra-orphan indications

Orphan Diseases (affects <200,000 people)



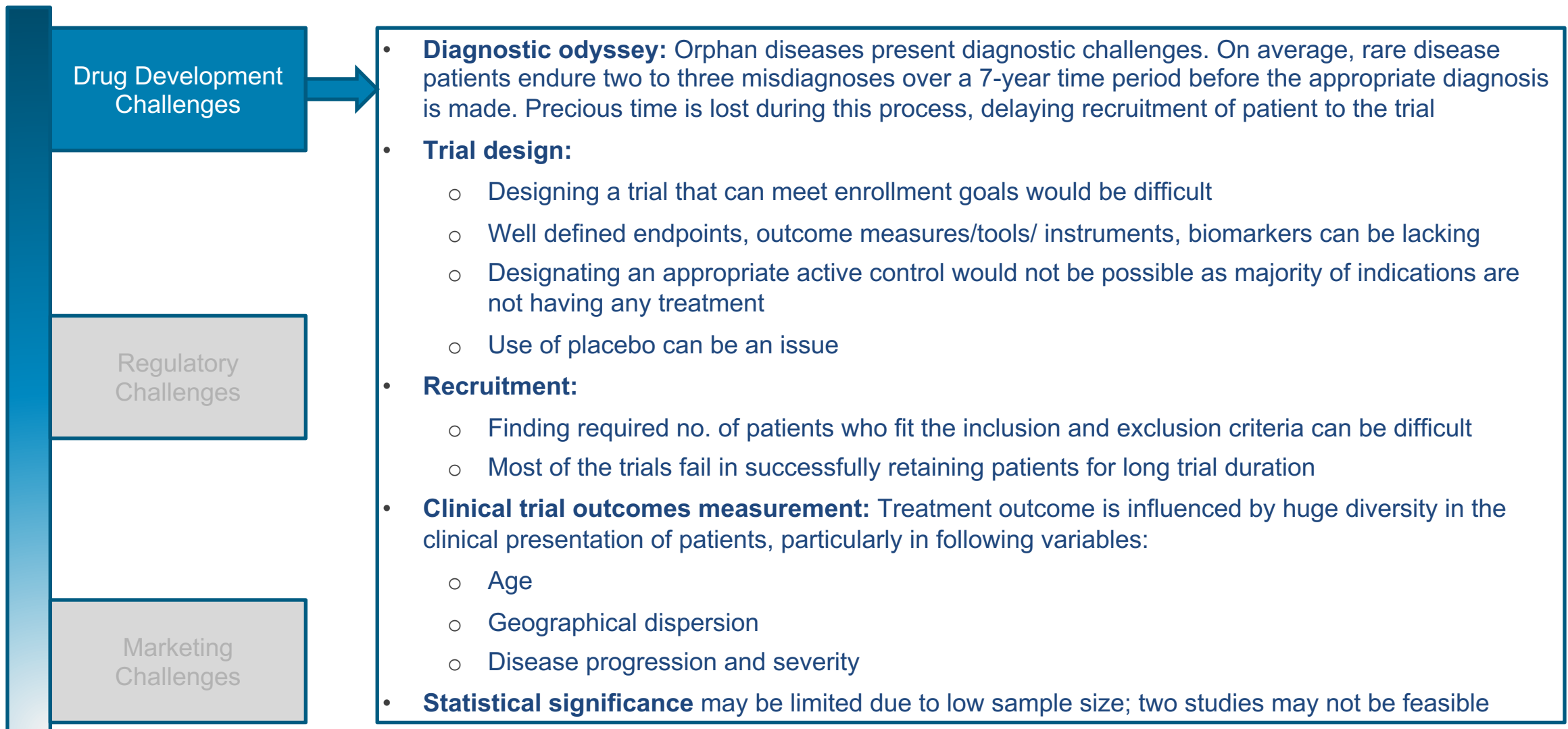
Disease	Incidence in U.S.
Pancreatic cancer	~60,000
Biliary tract cancer	~ 23,000
AML	~20,000
T-cell lymphoma	~12,000

Ultra-orphan Diseases (affects <10,000 people)

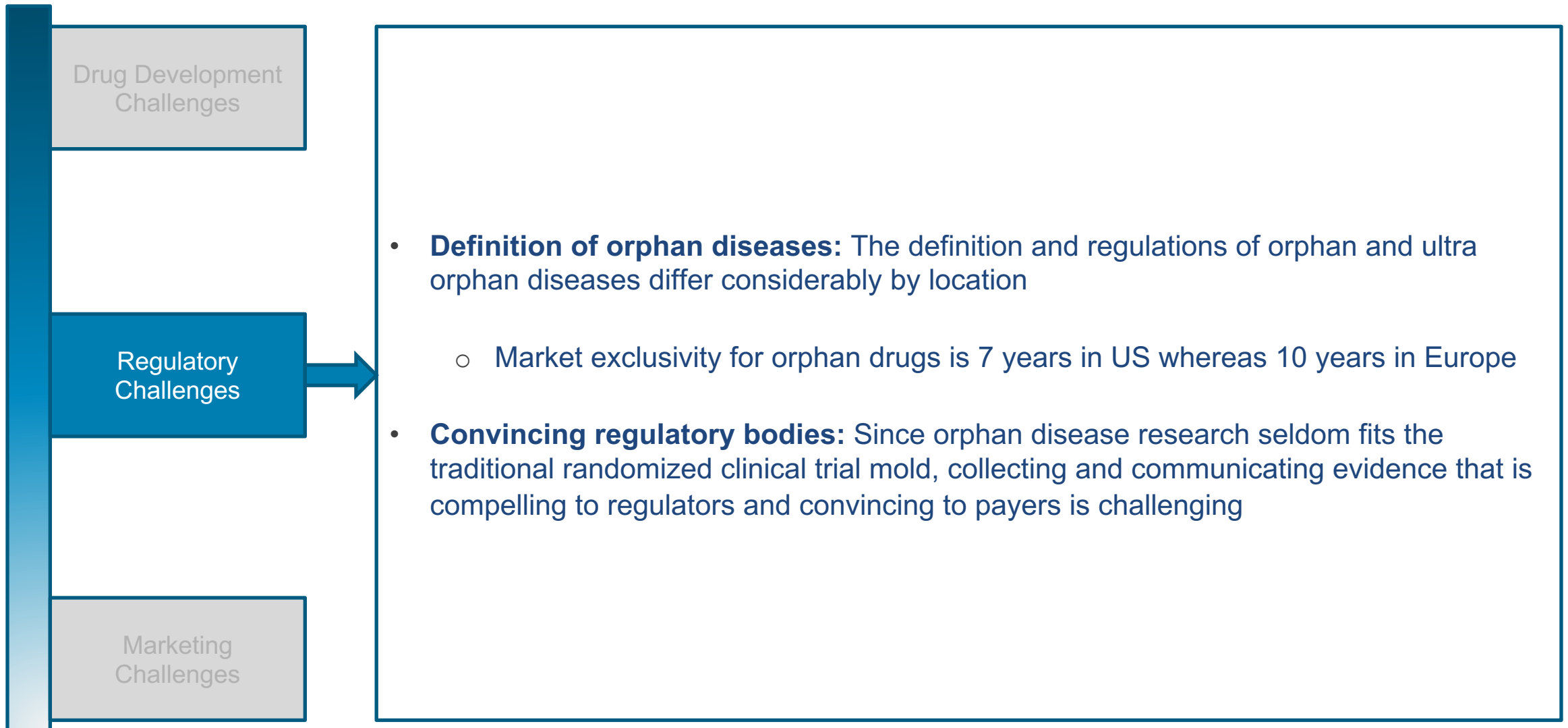


Disease	Incidence in U.S.
MDS	~10,000
Burkitt's lymphoma	~1,200
Clear cell sarcoma	~1,000

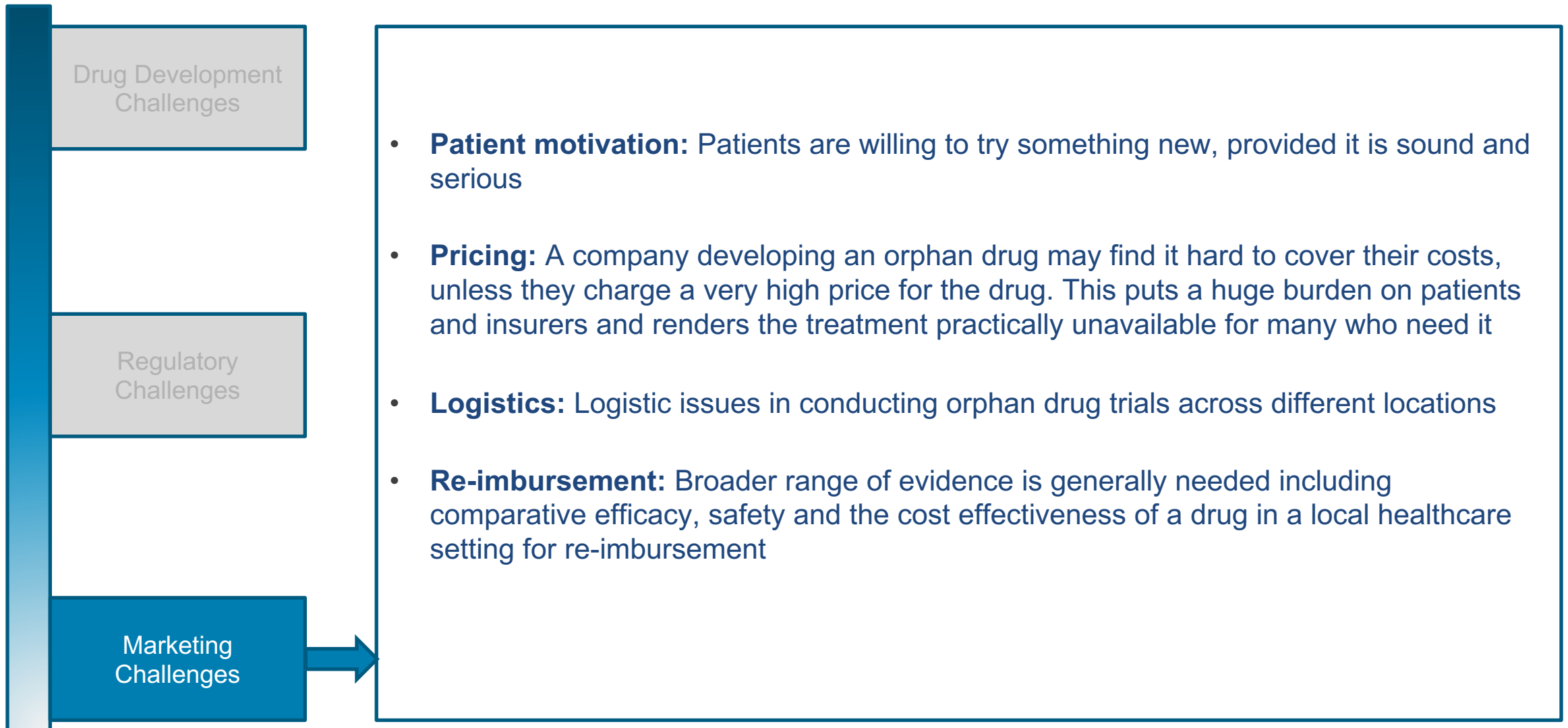
Orphan / Ultra-orphan Indications – Challenges



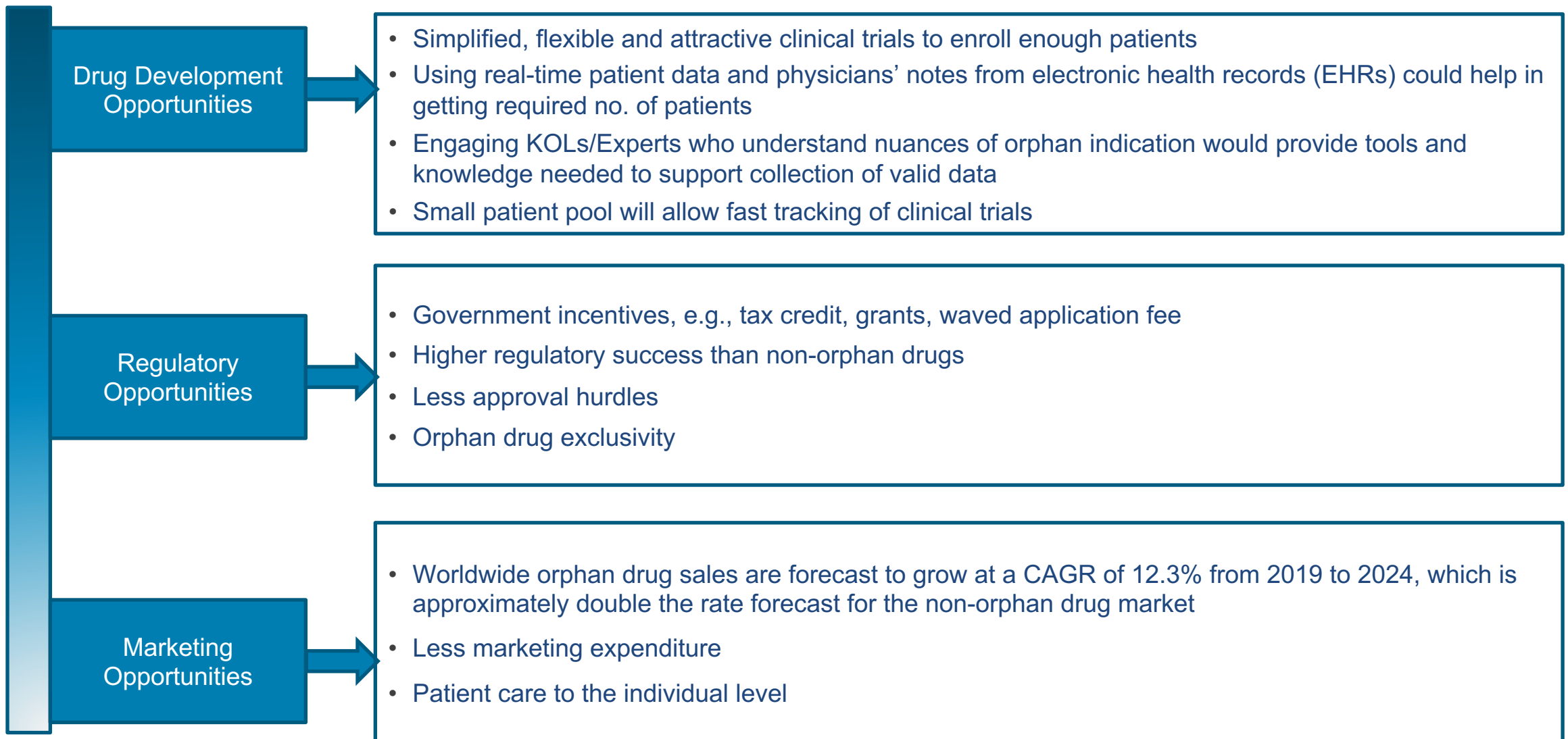
Orphan / Ultra-orphan Indications – Challenges



Orphan / Ultra-orphan Indications – Challenges



Orphan / Ultra-orphan Indications – Opportunities



Relationships Remain Central

- ❖ **Relationship** with front-line clinical researchers, investigators at academic sites
- ❖ Finding the specialists who are **passionate about rare cancers**



- ❖ **Relationships** with the **patient advocacy groups**: for the development of our drug in **Burkitt's lymphoma**, we have developed relationship with patient advocacy group and for **clear cell sarcoma**, we collaborated with **Sara's Cure**
- ❖ Ensuring there is a **patient population in the area** where we have the trial



- ❖ **Relationships** with the **regulatory bodies**: sharing the need for treatment options with the FDA



Starting from the Top Down for Clear Cell Sarcoma Patients

Top: Patients

Patient advocacy group
identified research from Japan

Middle: Rafael

Connected with patient
advocacy group and saw the
need

Bottom: FDA

Partnership with the
FDA to begin trials

A scientist in a white lab coat, white face mask, and safety glasses is using a pipette to transfer liquid into small vials. Another scientist is visible in the background, also wearing a mask and safety glasses. The scene is set in a bright laboratory with large windows.

***Determining
where to point
the laser***



RAFAEL
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Orphan / Ultra-orphan Indications: Rafael Advantage



Laser focus on orphan/ultra-orphan diseases



Novel and safe approach to cancer treatment



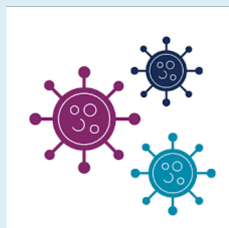
Targeting Multiple orphan indications with high unmet need



Pushing hard to include minorities in trials



Robust pipeline covering multiple orphan indications



Only company with a study in Burkitt's lymphoma and among few companies with a study in CCS



Collaboration with investigators, sites and patient efficacy groups



Broader/deeper penetration of Phase 3 pivotal studies (PANC & AML) across countries to get right population



Only oncology-focused company with 7 Orphan drug designations

“Extending and enhancing the lives of patients with cancer”



Questions? Submit on Swapcard! Thank You.