

# OCEAN Topline Data Webcast

May 25, 2021

# Participants



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On 26 February 2021, the U.S. Food and Drug Administration (“FDA”) approved PEPAXTO® (melphalan flufenamide, also known as melflufen), in combination with dexamethasone, for the treatment of adult patients with relapsed or refractory multiple myeloma, who have received at least four prior lines of therapy and whose disease is refractory to at least one proteasome inhibitor, one immunomodulatory agent, and one CD38-directed monoclonal antibody. This indication has been granted under accelerated approval based upon data from the HORIZON study. Melflufen is not approved by any other registration authorities.

Melflufen is an abbreviated form of the international non-proprietary name (INN) melphalan flufenamide

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# OCEAN topline webcast – Key take aways

- OCEAN – BOLDLY designed study with a SUCCESSFUL outcome
  - First positive Head-to-Head study in multiple myeloma in 6 years
- PEPAXTO demonstrated ~40% higher mPFS to pomalidomide, the most widely used drug in RRMM
  - Pomalidomide sales over \$3 billion worldwide, growing at 20%+ annually
- PEPAXTO on a path to be a foundational treatment in RRMM
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*Thanks to the patients, investigators, investors and Oncopeptides Team that made this trial possible*

**OCEAN TRIAL IS POSITIVE!!!**



**Celebrating a major achievement for RRMM patients**

# OCEAN Topline Webcast - Agenda



R&D Strategy | Our development plan and OCEAN rationale – *Jakob Lindberg*



OCEAN | Topline data, communication of full results, and regulatory plan – *Klaas Bakker*



Commercial opportunity and future plans – *Marty Duvall*



Q&A – *Oncopeptides Team*

# OCEAN overview – reflections on trial design



- Vast majority of clinical trials in oncology are add-on trials
- The dominance is such that most developers and clinicians instinctively interpret results – regardless of design – through the lens of add-on trials
- This poses a challenge when communicating head-to-head clinical trial results despite being the preferred design from a clinical relevance point of view
- Placebo-controlled trials are not acceptable in oncology due to the severity of the disease.
  - Single-arm trials are conducted if there are no treatment options

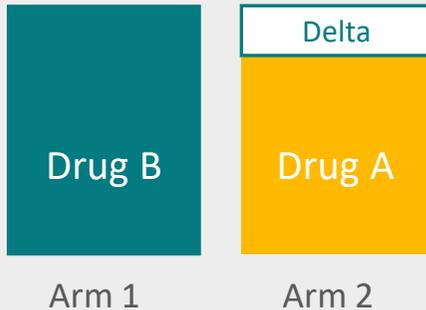
# Head-to-Head versus Add-on trials

## A comparison



### Head-to-Head

The trial measures the delta effect of Drug B compared to Drug A (safety and efficacy)



### Add-on

The trial measures the additive effect of Drug B (safety and efficacy)



# OCEAN overview – A Head-to-Head Comparison



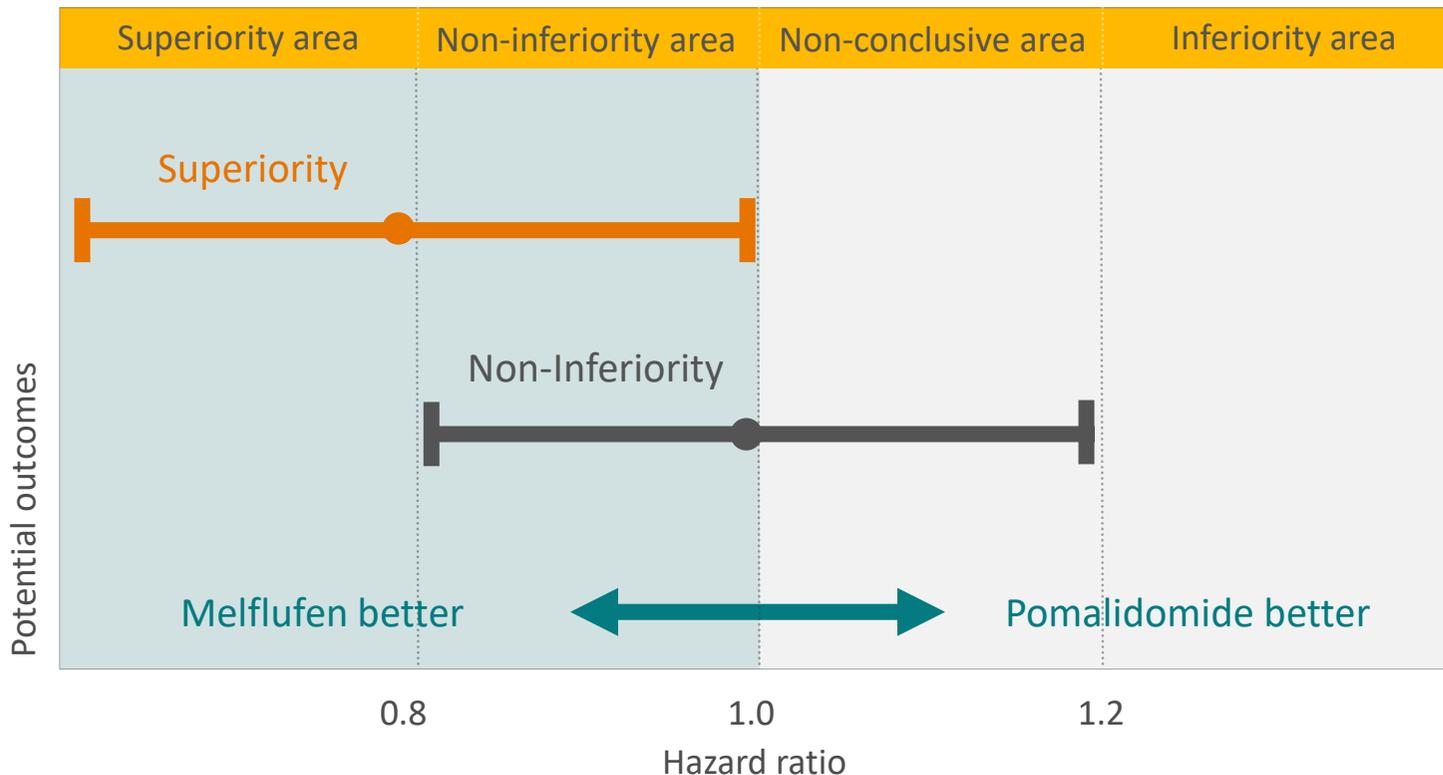
- Successful drugs in MM all have labels that allow for single-agent use (+/- steroid)
- Drivers for this are patient differences in tolerability, co-morbidities and refractory status
  - Almost half the patients are still only receiving single-agents (+/- steroid)
  - Significant off-label combination use
- In 2015/2016, the FDA did not see a medical need population in MM any longer (post daratumumab approval) and did not accept high-dose dexamethasone as a comparator in a randomized setting
- A single-agent (+/- steroid) label was only achievable through a head-to-head comparison with either lenalidomide, bortezomib or pomalidomide

# Two ways to be successful in OCEAN

## Head-to-head study with pomalidomide

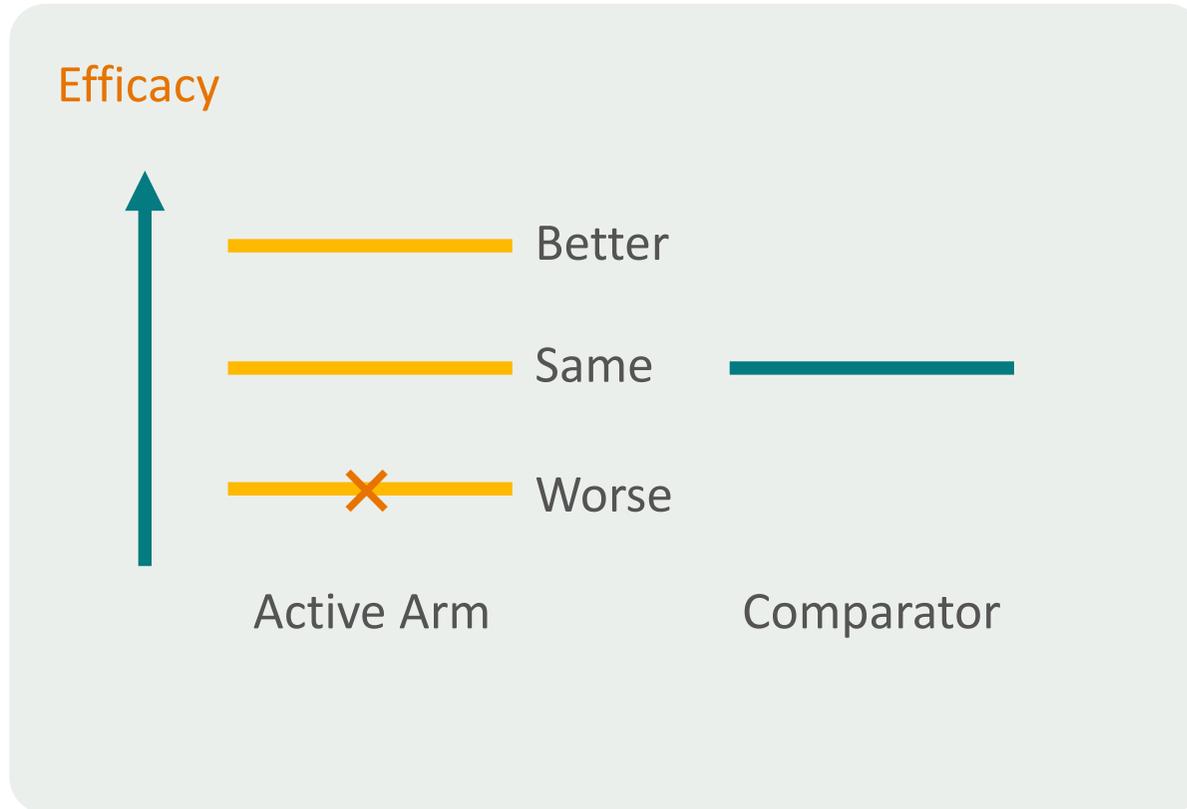


Successful OCEAN trial would have a superior or non-inferior result



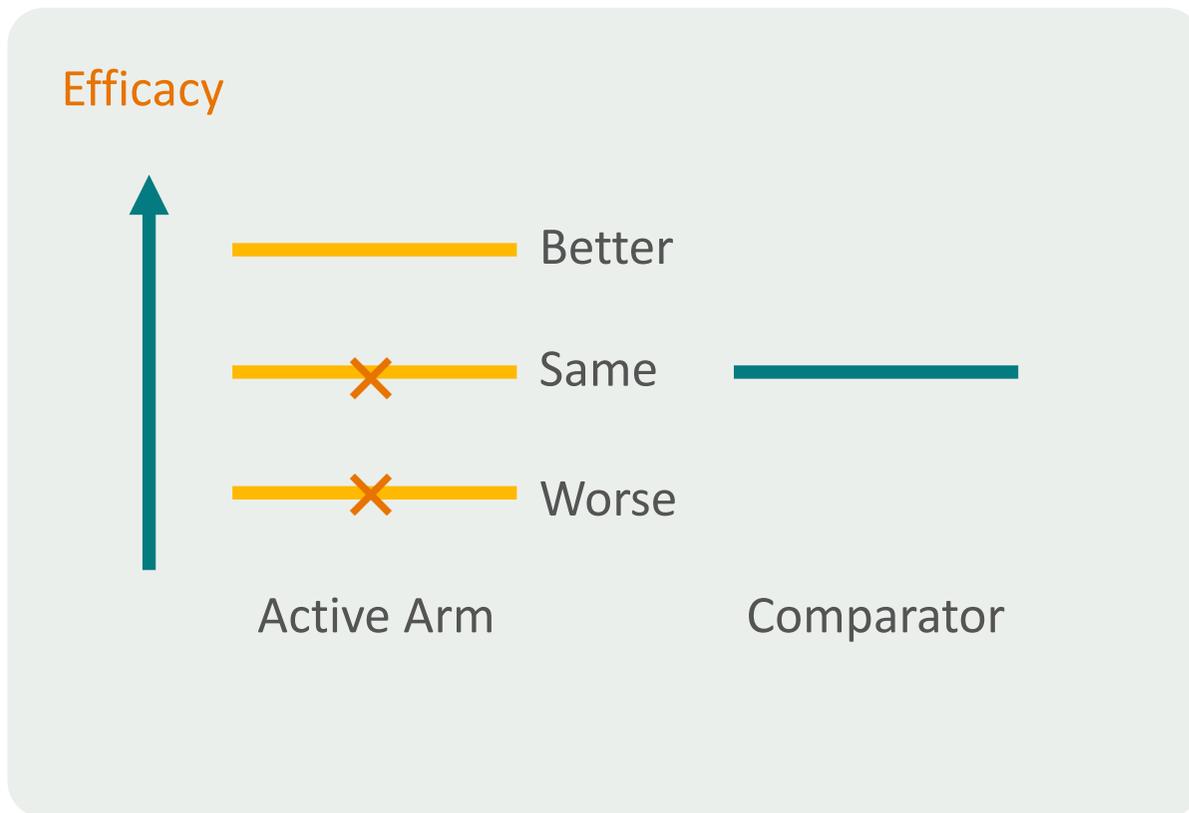
# Clinical trial outcome

– Non-inferiority



# Clinical trial outcome

– Superiority



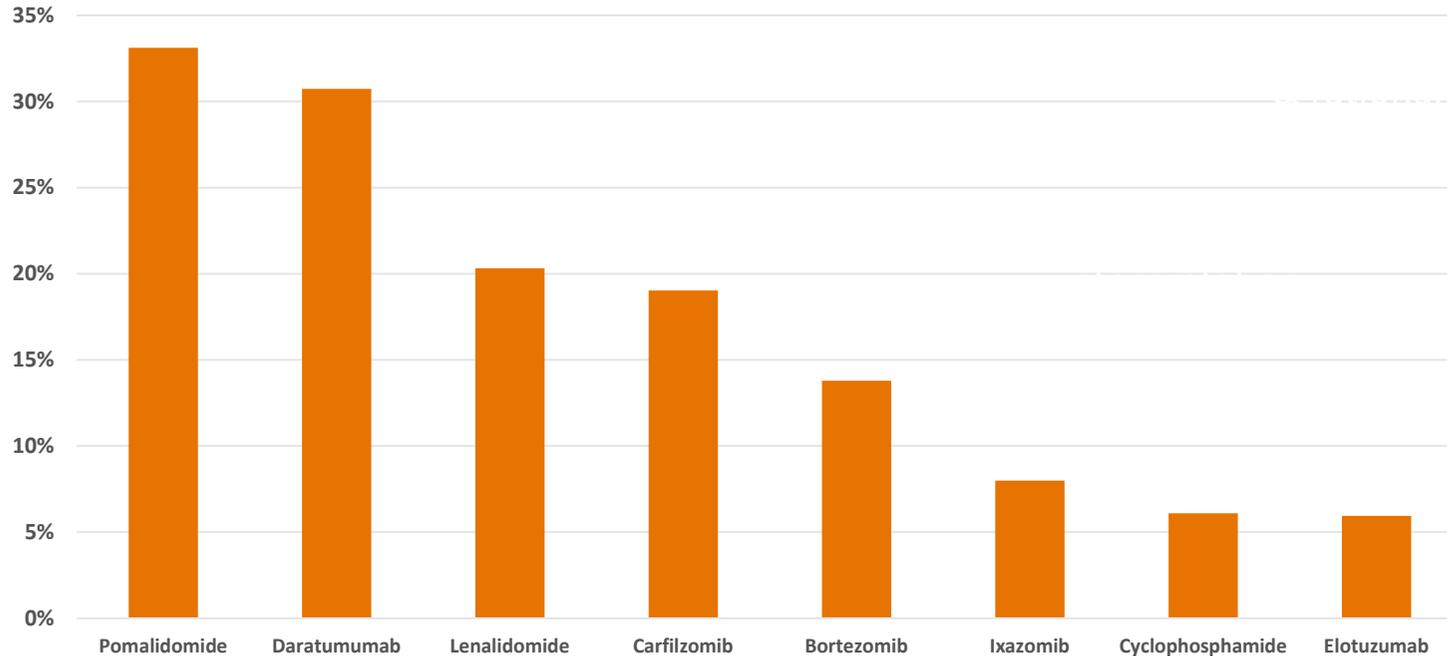
# Why a Head-to Head study with pomalidomide?

Pom is the most prescribed drug in RRMM (defined as 3L+)



## US RRMM Patient Market Share 2020 (%)

Drugs with more than 5% share



Pomalidomide is the most prescribed drug in RRMM and the key reference point for safety and efficacy

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Q&A– *Oncopeptides Team*

# Label expansion opportunity with phase 3 OCEAN study

## Confirmatory global study in 100+ sites in 21 countries



### Head-to-head study versus pomalidomide

Patients have failed 2-4 lines prior therapy, including refractory to lenalidomide within 18 months or have progressed on lenalidomide within 60 days of randomization

**N = 495**  
Lenalidomide-refractory multiple myeloma patients

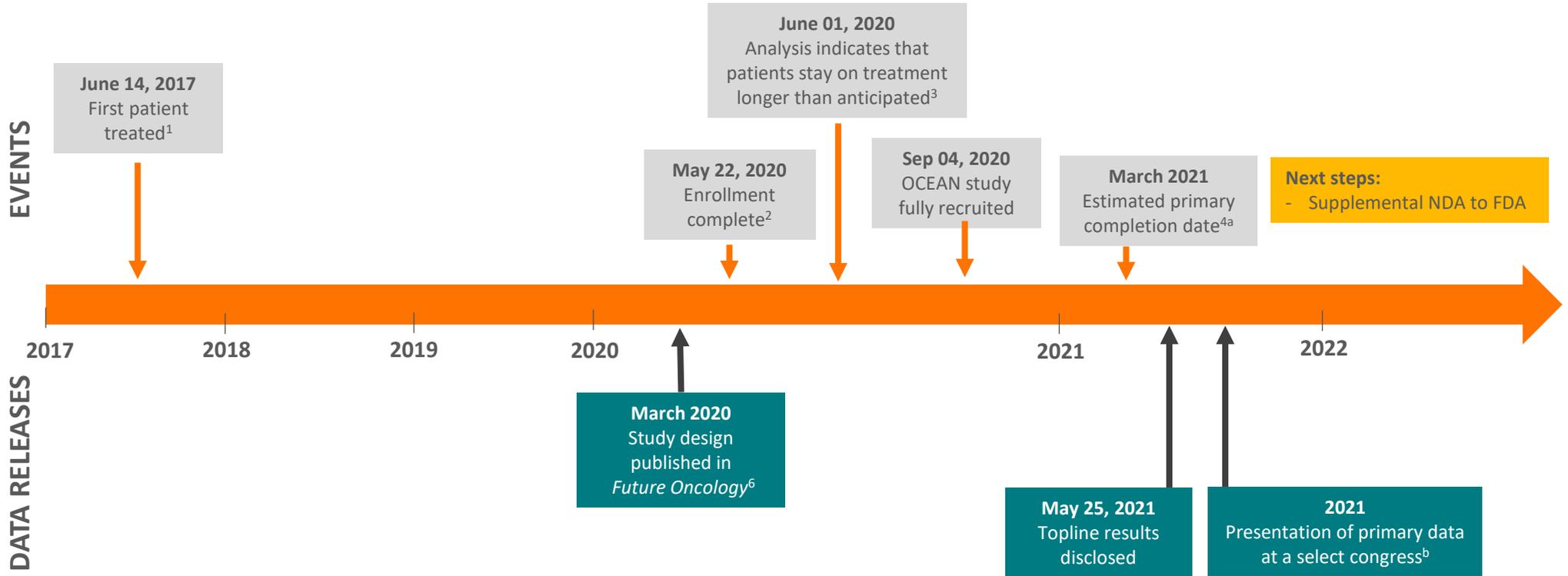
Melflufen +  
dexamethasone

Pomalidomide +  
dexamethasone

Primary  
endpoint:  
PFS

Secondary  
endpoint:  
ORR, OS

# OCEAN study – detailed timeline



<sup>a</sup>Event-driven; <sup>b</sup>Current assumption and plan.

1. Oncopeptides [Press Release](#), June 14, 2017; 2. Oncopeptides [Press Release](#), May 22, 2020; 3. Oncopeptides [Press Release](#), June 01, 2020; 4. ClinicalTrials.gov Identifier: [NCT03151811](#); 5. Sonneveld P, et al. [Poster Presentation P-036] Lymphoma & Myeloma Congress 2019; 6. Schjesvold F, et al. *Future Oncol.* 2020;16:631–641. 7. Oncopeptides [Press Release](#), Sep 04, 2020

# OCEAN DATA – Topline results



- Primary endpoint – Progression Free Survival (PFS)

	Hazard Ratio (95% CI)	P-Value	Relative mPFS improvement	Outcome
Independent Review Committee (IRC)	0.817 (0.659-1.012)	0.064	+41%	Non-Inferiority
Investigator Assessed Results	0.790 (0.639-0.976)	0.029	+42%	Superiority

- Overall Response Rate 32.1% for melflufen vs 26.5% for pomalidomide

## OCEAN DATA – Safety summary



- Safety profile of melflufen was in line with previous studies
- Pomalidomide had slightly more infections than melflufen
- Similar levels of other non-hematologic toxicities were observed
- Discontinuation rates for AEs were similar in both arms

# What does that mean for engaging FDA and US PEPAXTO label?

We are currently in the process of engaging FDA on the OCEAN data

Presentations at key conferences

Publication in progress



We plan to file for supplementary NDA in Q4 2021



In light of the OCEAN trial results, we plan to ask for:

- Label change (new indication)
- Full approval (fulfill requirements for the accelerated approval)



We continue and focus on our commercialization efforts with PEPAXTO in the US



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# OCEAN and LIGHTHOUSE opportunity

## OCEAN

Pomalidomide is a \$3.1b product in a \$13b RRMM market

~\$600m sales in 2020 or ~40% of Pom use is attributed to PomDex doublet use in RRMM

A positive OCEAN trial stands to gain share in the 3L+ setting, taking from PomDex and other doublet therapies, with doublet use making up a majority (~55%) of 3L+ treatments



## LIGHTHOUSE

Darzalex has grown quickly into a \$4.2b product driven by increased combination use in the US

~\$600m sales in 2020 or ~40% of Pom use is attributed to DaraPomDex triplet use in RRMM

While majority of Dara is currently used in combination regimens with other agents, single agent Dara is used in 5-10% of RRMM in the US

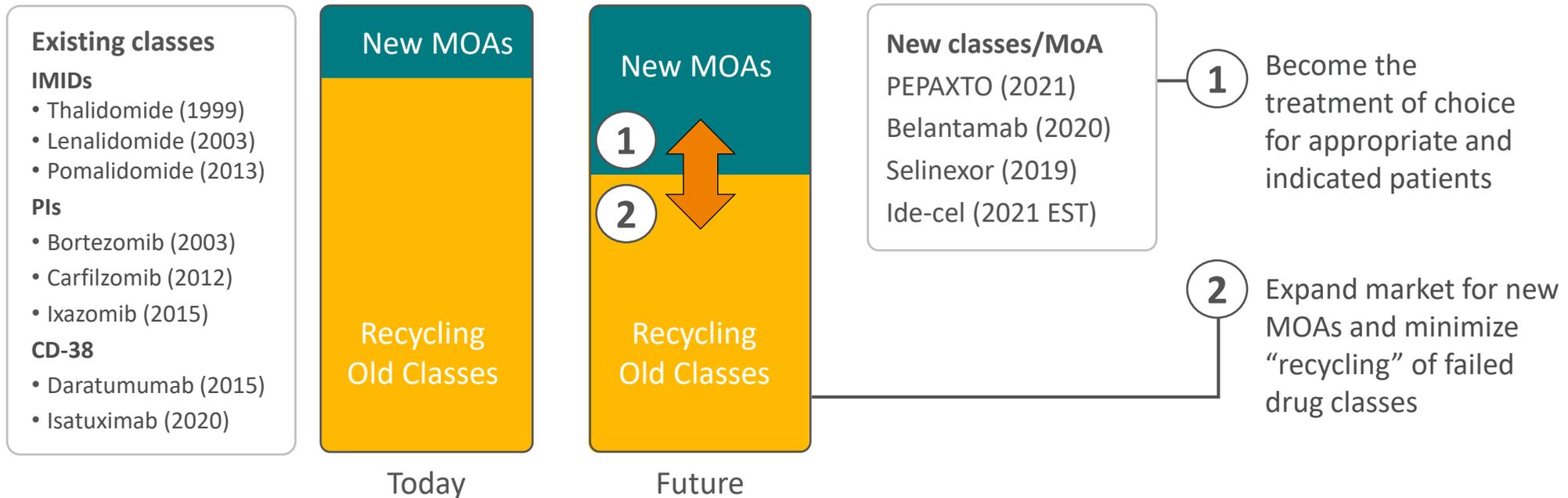


Our trials are linked to the largest drugs WW in MM

# PEPAXTO strategy - Two-pronged approach

## Becoming a foundational treatment in RRMM

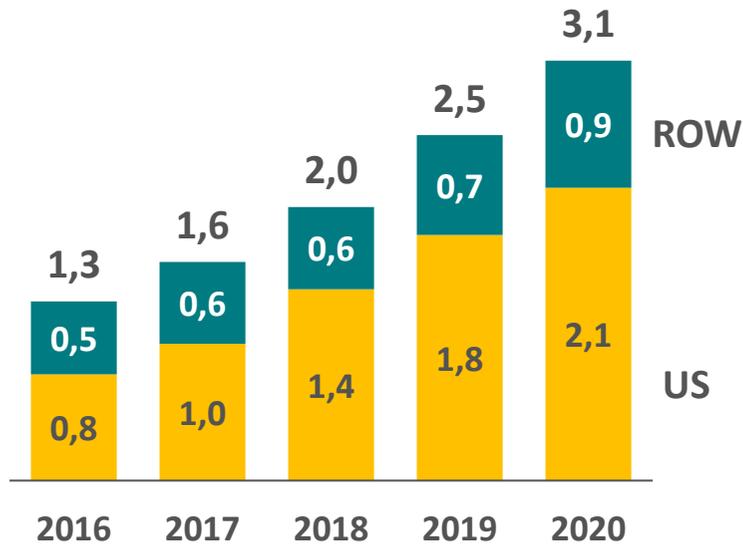
Driving change in today's RRMM treatment paradigm where drug classes are "recycled"



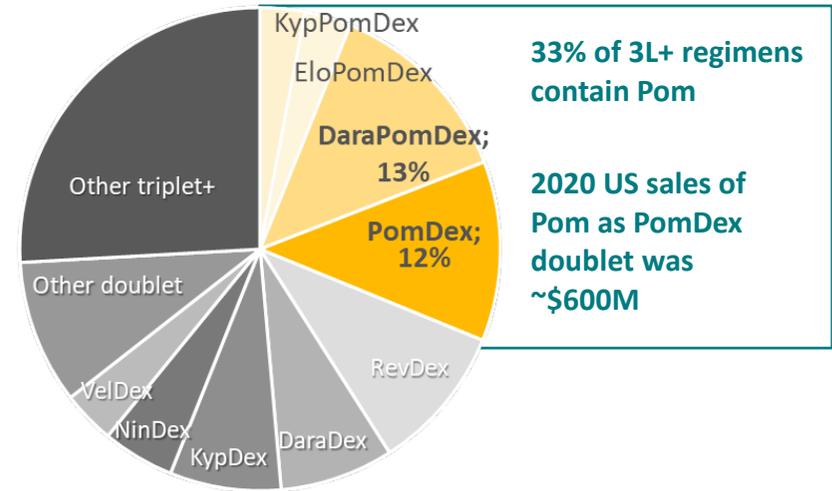
# Pomalidomide is the largest drug in RRMM

PomDex and PomDex combos comprising 33% of US share

### Pomalidomide Worldwide Sales (\$ Billions)

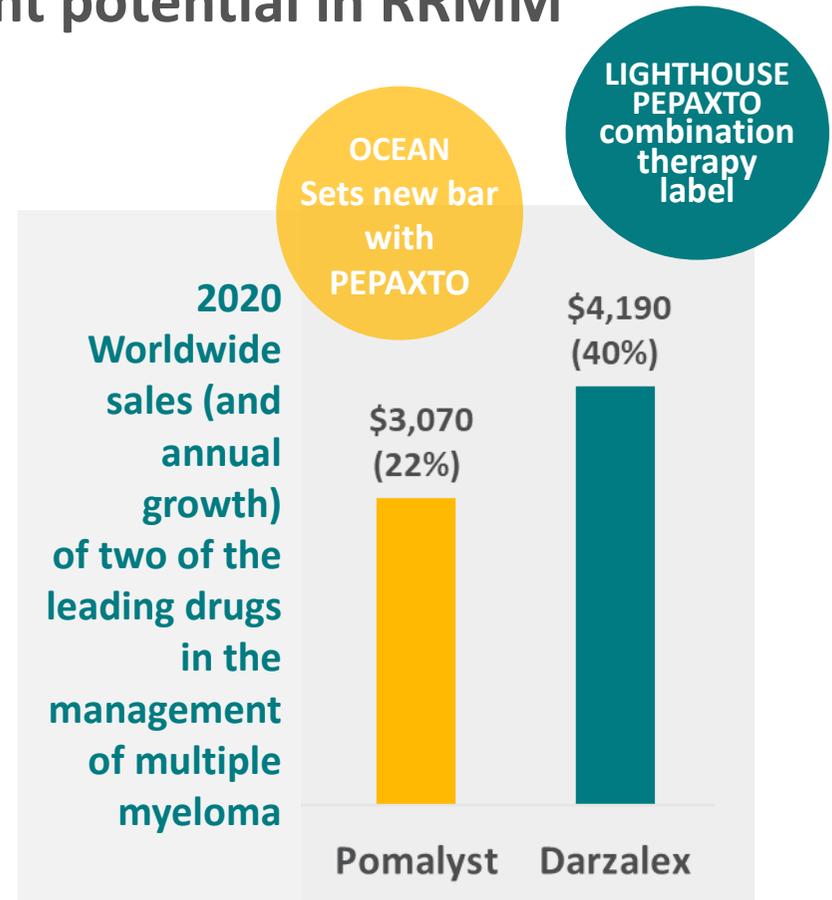


### US 2020 - 3L+ Patient Market Share Intrinsic Data



# PEPAXTO's emerging profile has significant potential in RRMM

- OCEAN – Sets new bar with PEPAXTO with positive head-to-head data
  - Strong efficacy profile as a doublet in 3<sup>rd</sup> and 4<sup>th</sup> line
- LIGHTHOUSE – First opportunity to expand label as part of a triplet regimen in RRMM
  - Opportunity to establish data in combination with the other workhorse drug in multiple myeloma



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bringing hope through science