

# OCEAN Topline Data Webcast

May 25, 2021

# Participants



**Marty J Duvall**  
Chief Executive Officer



**Jakob Lindberg**  
Chief Scientific Officer



**Klaas Bakker**  
Chief Medical Officer



**Anders Martin-Löf**  
Chief Financial Officer

# Disclaimer

**IMPORTANT:** You must read the following before continuing. The following applies to this document, the oral presentation of the information in this document by Oncopeptides AB (the “Company”) or any person on behalf of the Company, and any question-and-answer session that follows the oral presentation (collectively, the “Information”).

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

The Information contains forward-looking statements. All statements other than statements of historical fact included in the Information are forward-looking statements. Forward-looking statements give the Company’s current expectations and projections relating to its financial condition, results of operations, plans, objectives, future performance and business. These statements may include, without limitation, any statements preceded by, followed by or including words such as “target,” “believe,” “expect,” “aim,” “intend,” “may,” “anticipate,” “estimate,” “plan,” “project,” “will,” “can have,” “likely,” “should,” “would,” “could” and other words and terms of similar meaning or the negative thereof. Such forward-looking statements involve known and unknown risks, uncertainties and other important factors beyond the Company’s control that could cause the Company’s actual results, performance or achievements to be materially different from the expected results, performance or achievements expressed or implied by such forward-looking statements. Such forward-looking statements are based on numerous assumptions regarding the Company’s present and future business strategies and the environment in which it will operate in the future.

No representation, warranty or undertaking, express or implied, is made as to, and no reliance should be placed on, the fairness, accuracy, completeness or correctness of the Information or the opinions contained therein. The Information has not been independently verified and will not be updated. The Information, including but not limited to forward-looking statements, applies only as of the date of this document and is not intended to give any assurances as to future results. The Company expressly disclaims any obligation or undertaking to disseminate any updates or revisions to the Information, including any financial data or forward-looking statements, and will not publicly release any revisions it may make to the Information that may result from any change in the Company’s expectations, any change in events, conditions or circumstances on which these forward-looking statements are based, or other events or circumstances arising after the date of this document. Market data used in the Information not attributed to a specific source are estimates of the Company and have not been independently verified.

## 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
- Full data to be presented at a conference as soon as possible
- sNDA submission planned for in Q4 2021

*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

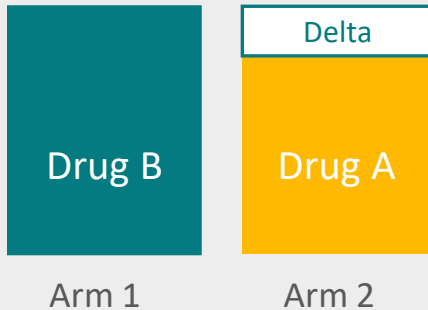
# Head-to-Head versus Add-on trials

## A comparison



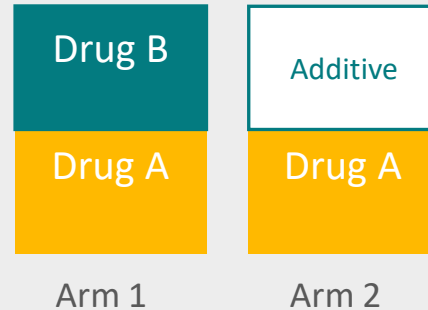
### Head-to-Head

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



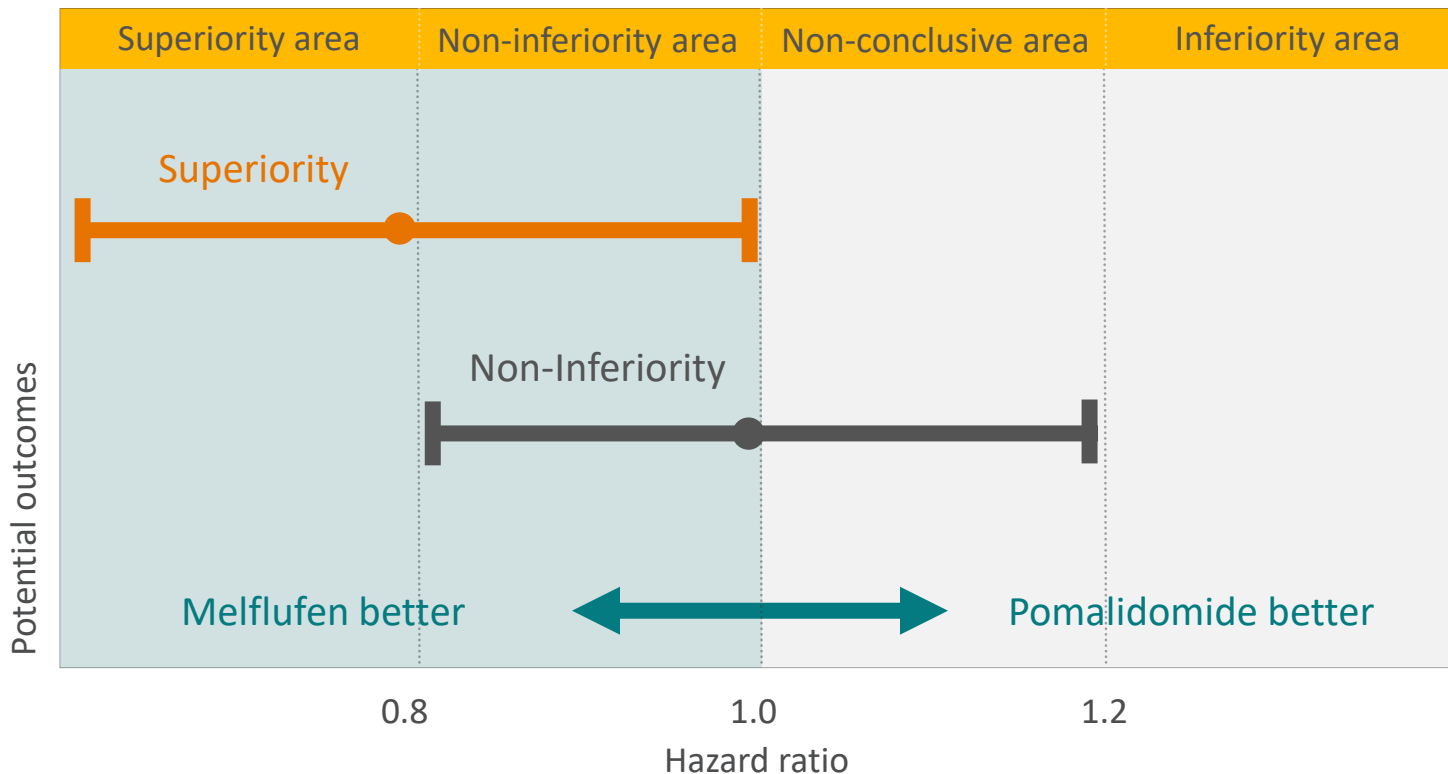
- Successful drugs in MM all have labels that allow for single-agent use (+/- steroid)
- Drivers for this are patient differences in tolerability, co-morbidities and refractory status
  - Almost half the patients are still only receiving single-agents (+/- steroid)
  - Significant off-label combination 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

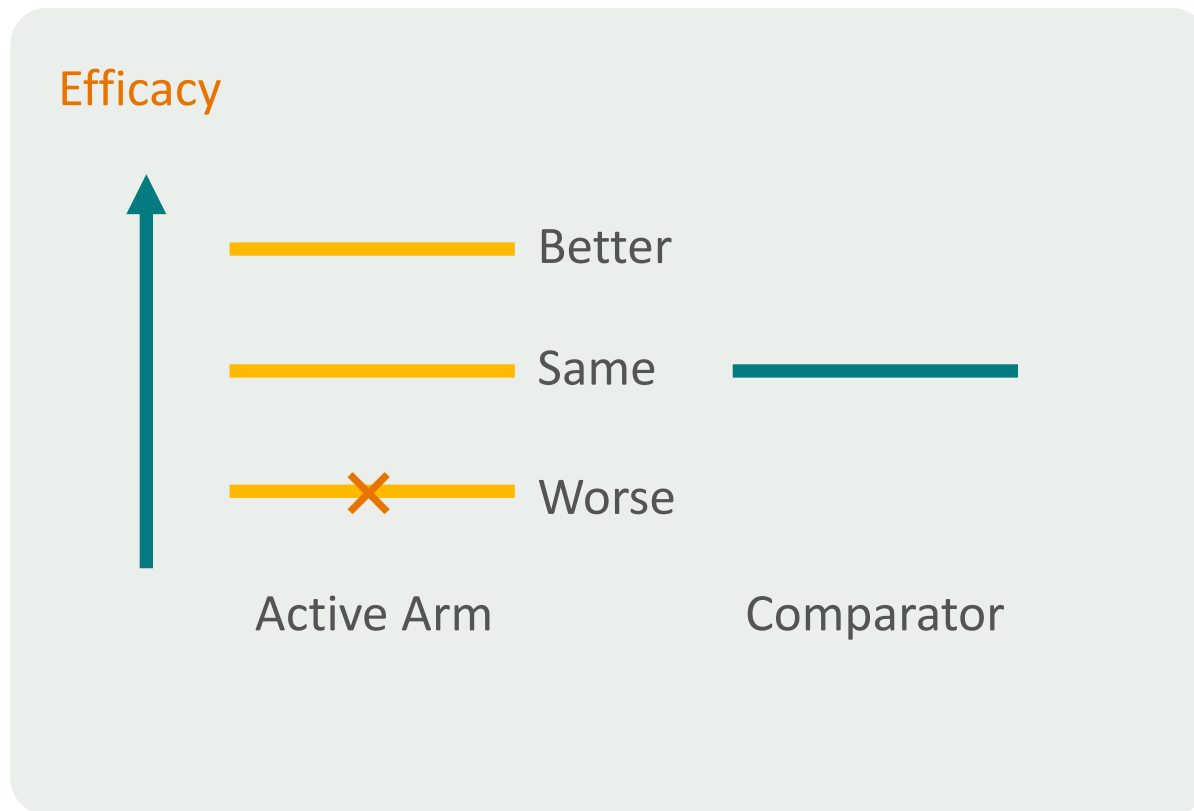


Successful OCEAN trial would have a superior or non-inferior result



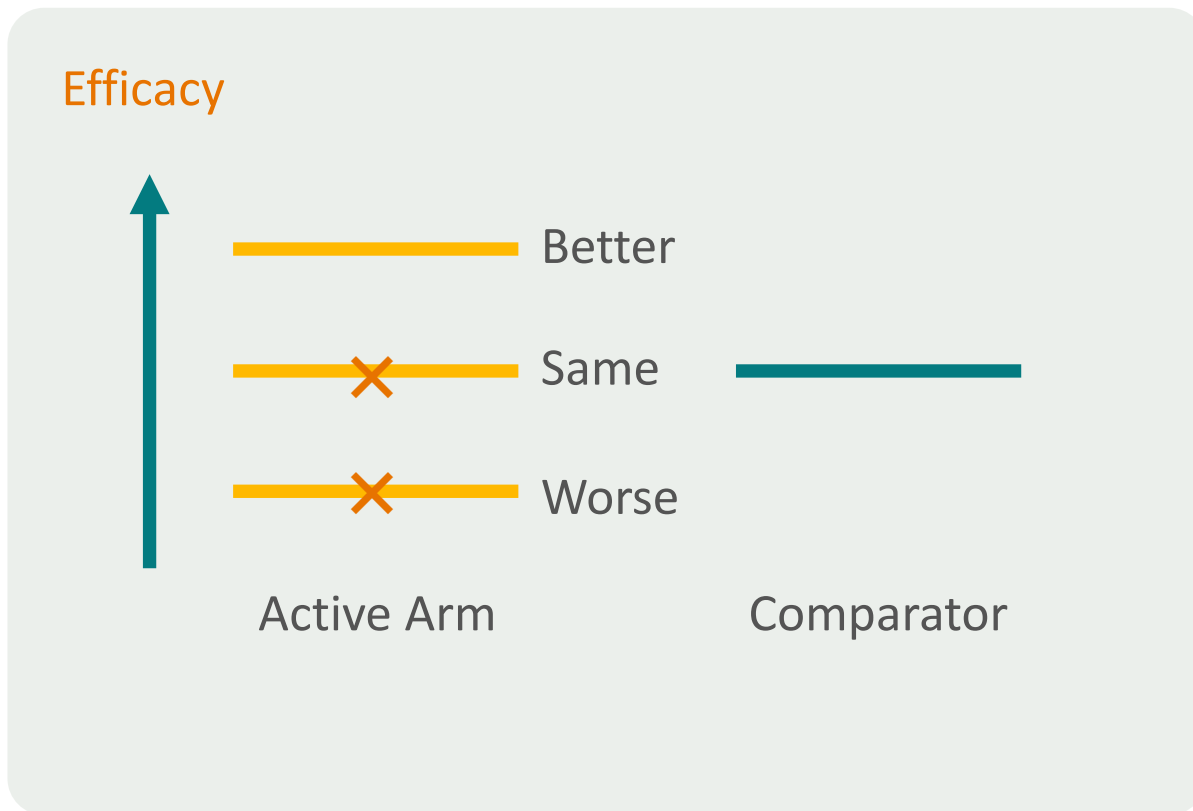
# Clinical trial outcome

– Non-inferiority



# Clinical trial outcome

– Superiority



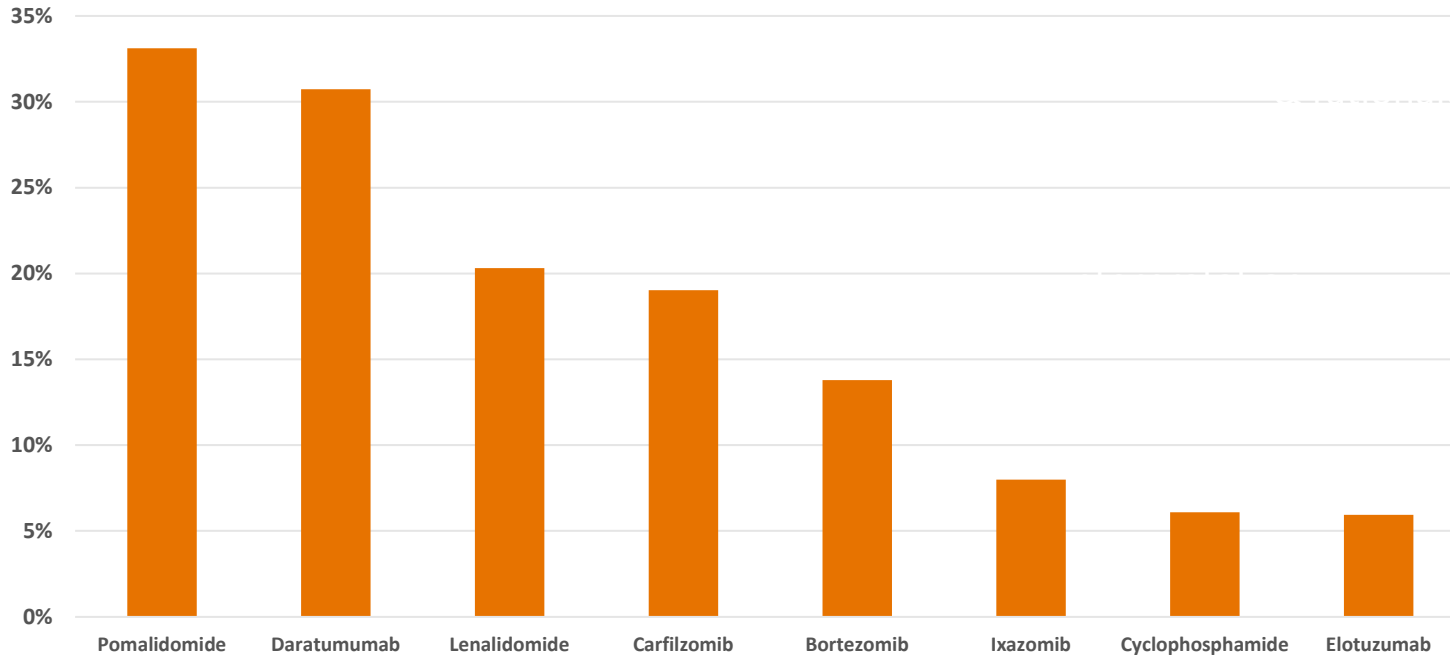
# Why a Head-to Head study with pomalidomide?

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

# 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*

# 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

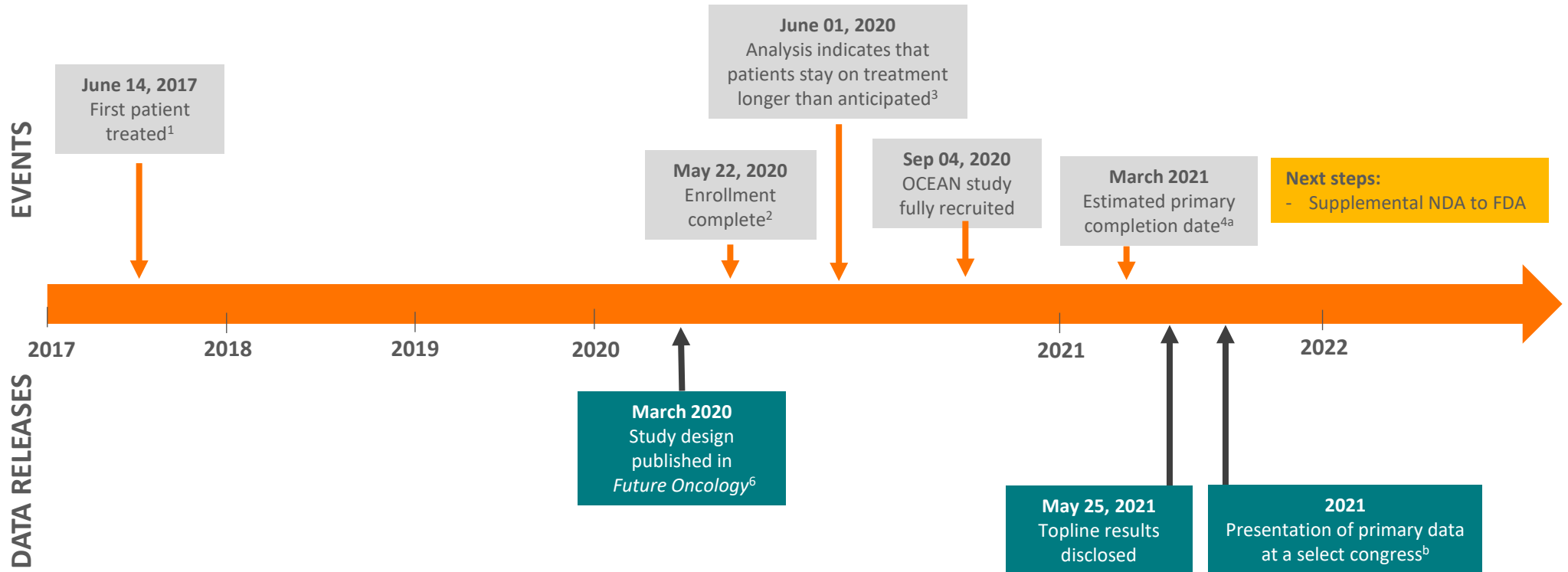
Melflufen +  
dexamethasone

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



# 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*



Questions and Answers – *Oncopeptides Team*

# 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

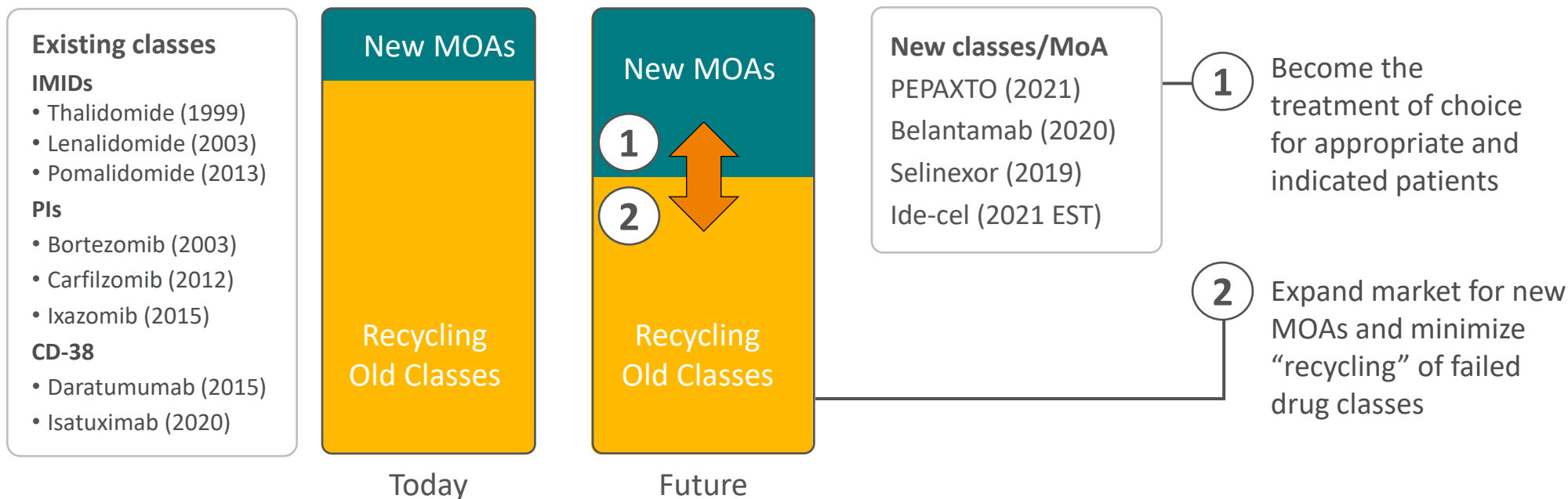


Our trials are linked to the largest drugs WW in MM

# PEPAXTO strategy - Two-pronged approach

## Becoming a foundational treatment in RRMM

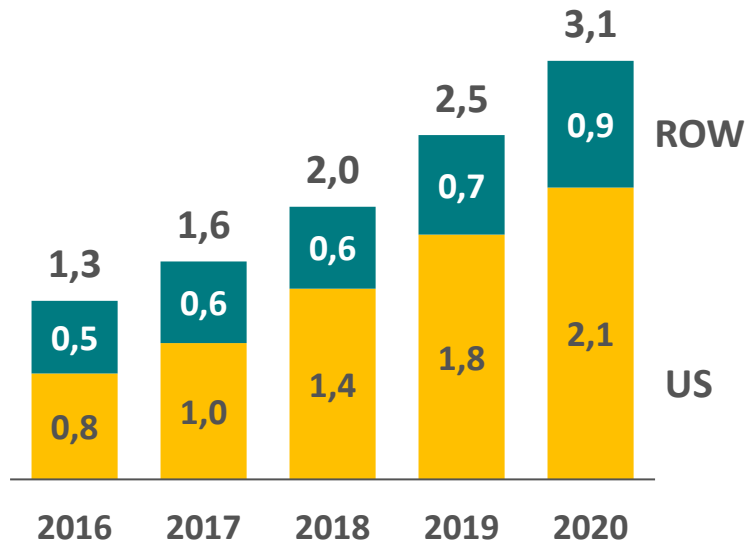
Driving change in today's RRMM treatment paradigm where drug classes are "recycled"



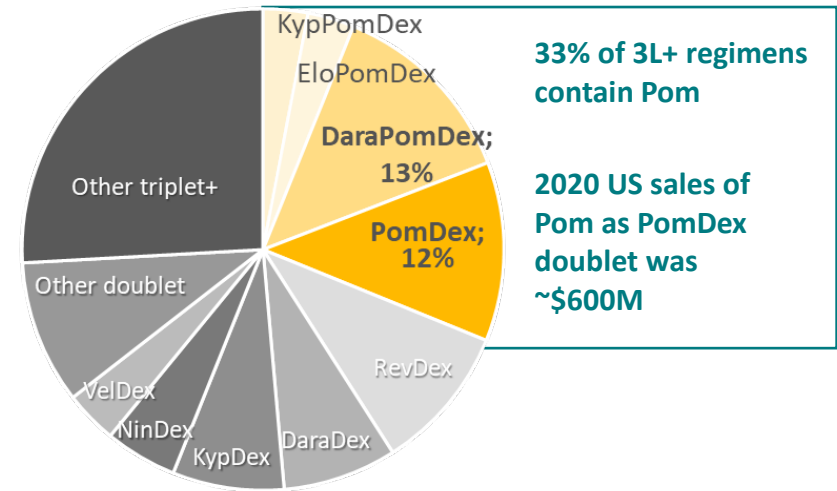
# Pomalidomide is the largest drug in RRMM

PomDex and PomDex combos comprising 33% of US share

### Pomalidomide Worldwide Sales (\$ Billions)

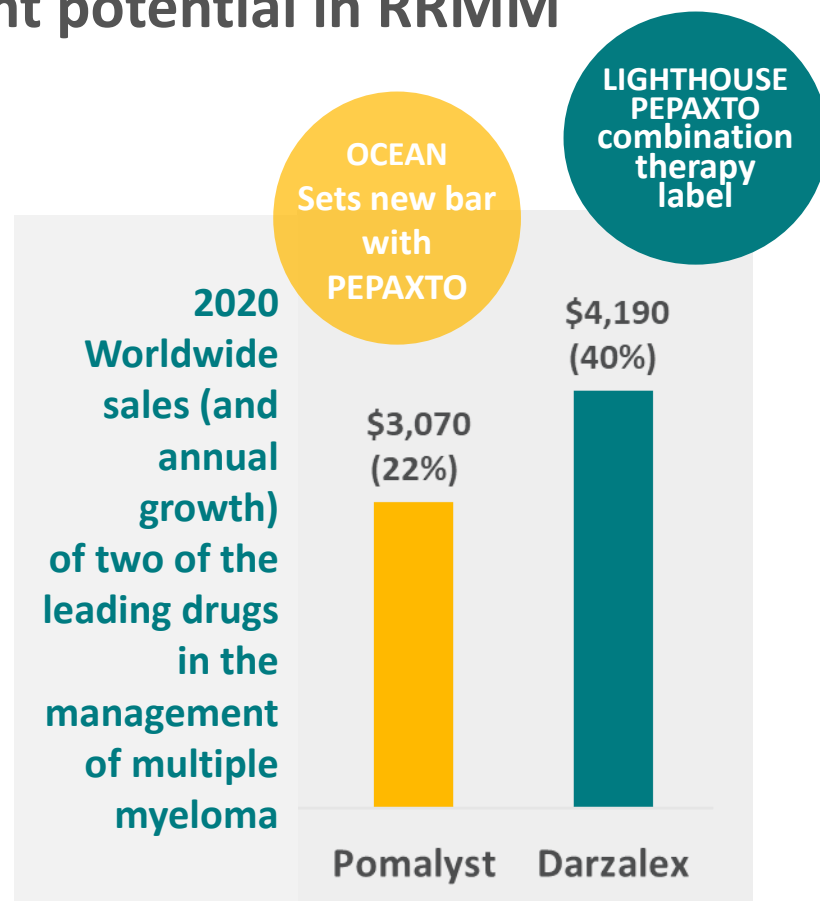


### US 2020 - 3L+ Patient Market Share Intrinsic Data



# 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



# 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
- Full data to be presented at a conference as soon as possible
- sNDA submission planned for in Q4 2021

*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*



Questions and Answers – *Oncopeptides Team*



oncopeptides

bringing hope through science