Cowen's 41st Annual Health Care Conference

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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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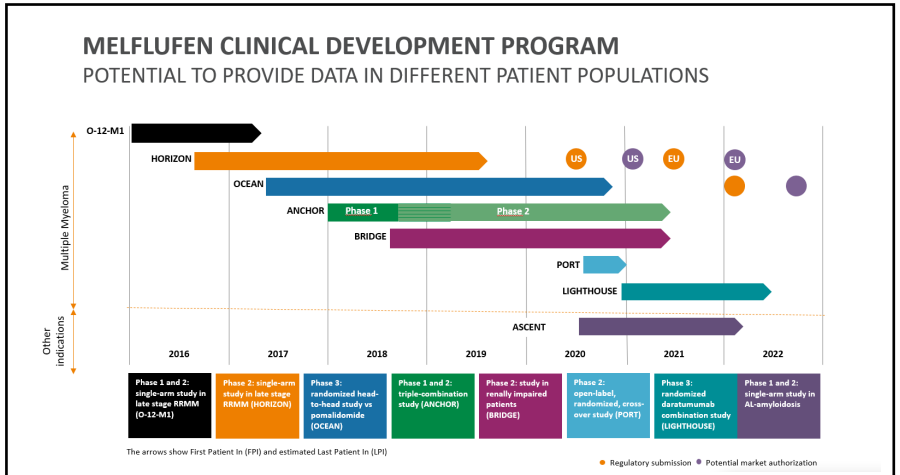
ONCOPEPTIDES AT A GLANCE

TRANSFORMATION INTO A FULLY INTEGRATED BIOTECH COMPANY

PEPAXTO® (melphalan flufenamide) NOW APPROVED BY THE FDA

Discovery and IND generation

- Targeted therapies for hematological diseases
- NCE:s from peptide drug conjugate platform (PDC)



Portfolio Development and Life Cycle Management

- Initial focus on $ 23 B MM market
- Broad supportive clinical program
- PEPAXTO® (melphalan flufenamide) now approved by the FDA

First commercial launch in US

Pepaxto® (melphalan flufenamide) injection for intravenous use 20 mg/vial

- Launching Pepaxto in the US during March
- Listed on NASDAQ Stockholm
- Market cap of ~ $ 1 580 M
- Cash position end of Q4 SEK 840 M (~ $ 100 M)

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HORIZON STUDY UNDERPINS THE FDA APPROVAL OF PEPAXTO

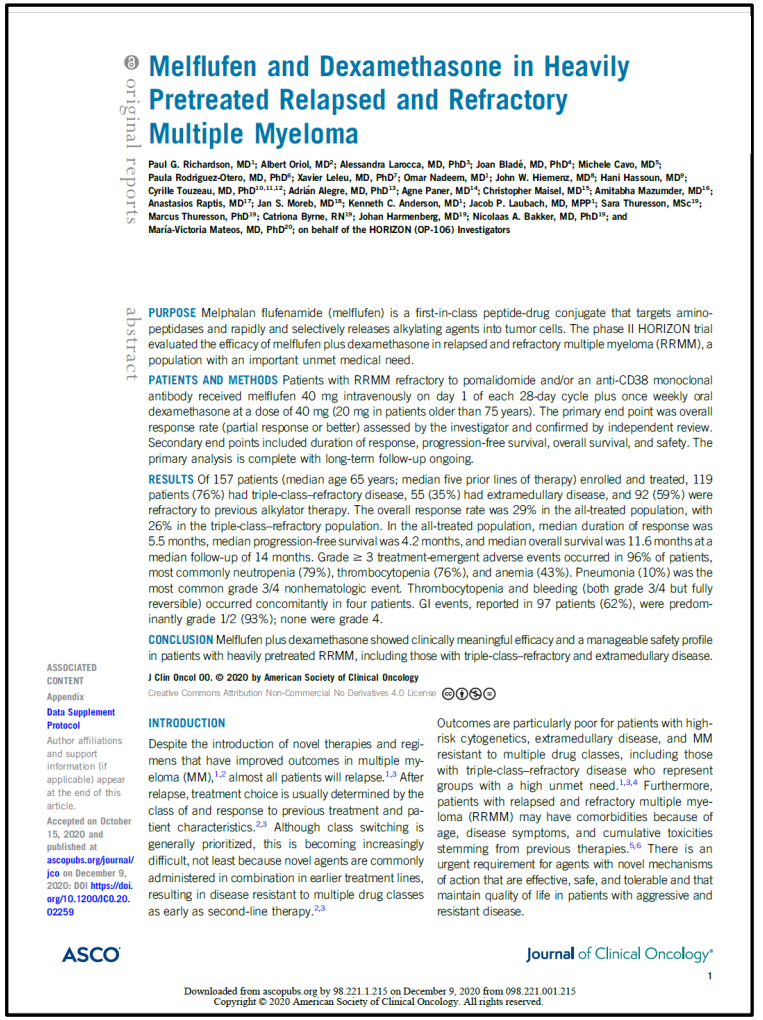
JOURNAL OF CLINICAL ONCOLOGY (DECEMBER 2020)

INCLUSION CRITERIA

- Adult multiple myeloma patients with documented disease progression
- At least 2 prior lines of therapy including an IMiD and a PI and a disease that at a minimum is refractory to pomalidomide and/or daratumumab

PATIENT INFORMATION

- 157 patients were recruited in total
- Median age – 65
- Median of 5 prior lines of therapy
- 76% of patients were triple-class refractory (or more)
- 59% of patients were refractory to previous alkylator therapy
- 35% of patients suffered from extramedullary disease (EMD)



original reports abstract

Melflufen and Dexamethasone in Heavily Pretreated Relapsed and Refractory Multiple Myeloma

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PURPOSE Melphalan flufenamide (melflufen) is a first-in-class peptide-drug conjugate that targets aminopeptidases and rapidly and selectively releases alkylating agents into tumor cells. The phase II HORIZON trial evaluated the efficacy of melflufen plus dexamethasone in relapsed and refractory multiple myeloma (RRMM), a population with an important unmet medical need.

PATIENTS AND METHODS Patients with RRMM refractory to pomalidomide and/or an anti-CD38 monoclonal antibody received melflufen 40 mg intravenously on day 1 of each 28-day cycle plus once weekly oral dexamethasone at a dose of 40 mg (20 mg in patients older than 75 years). The primary end point was overall response rate (partial response or better) assessed by the investigator and confirmed by independent review. Secondary end points included duration of response, progression-free survival, overall survival, and safety. The primary analysis is complete with long-term follow-up ongoing.

RESULTS Of 157 patients (median age 65 years; median five prior lines of therapy) enrolled and treated, 119 patients (76%) had triple-class–refractory disease, 55 (35%) had extramedullary disease, and 92 (59%) were refractory to previous alkylator therapy. The overall response rate was 29% in the all-treated population, with 26% in the triple-class–refractory population. In the all-treated population, median duration of response was 5.5 months, median progression-free survival was 4.2 months, and median overall survival was 11.6 months at a median follow-up of 14 months. Grade ≥ 3 treatment-emergent adverse events occurred in 96% of patients, most commonly neutropenia (79%), thrombocytopenia (76%), and anemia (43%). Pneumonia (10%) was the most common grade 3/4 nonhematologic event. Thrombocytopenia and bleeding (both grade 3/4 but fully reversible) occurred concomitantly in four patients. GI events, reported in 97 patients (62%), were predominantly grade 1/2 (93%); none were grade 4.

CONCLUSION Melflufen plus dexamethasone showed clinically meaningful efficacy and a manageable safety profile in patients with heavily pretreated RRMM, including those with triple-class–refractory and extramedullary disease.

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ASSOCIATED CONTENT

Appendix

Data Supplement

Protocol

Author affiliations and support information (if applicable) appear at the end of this article.

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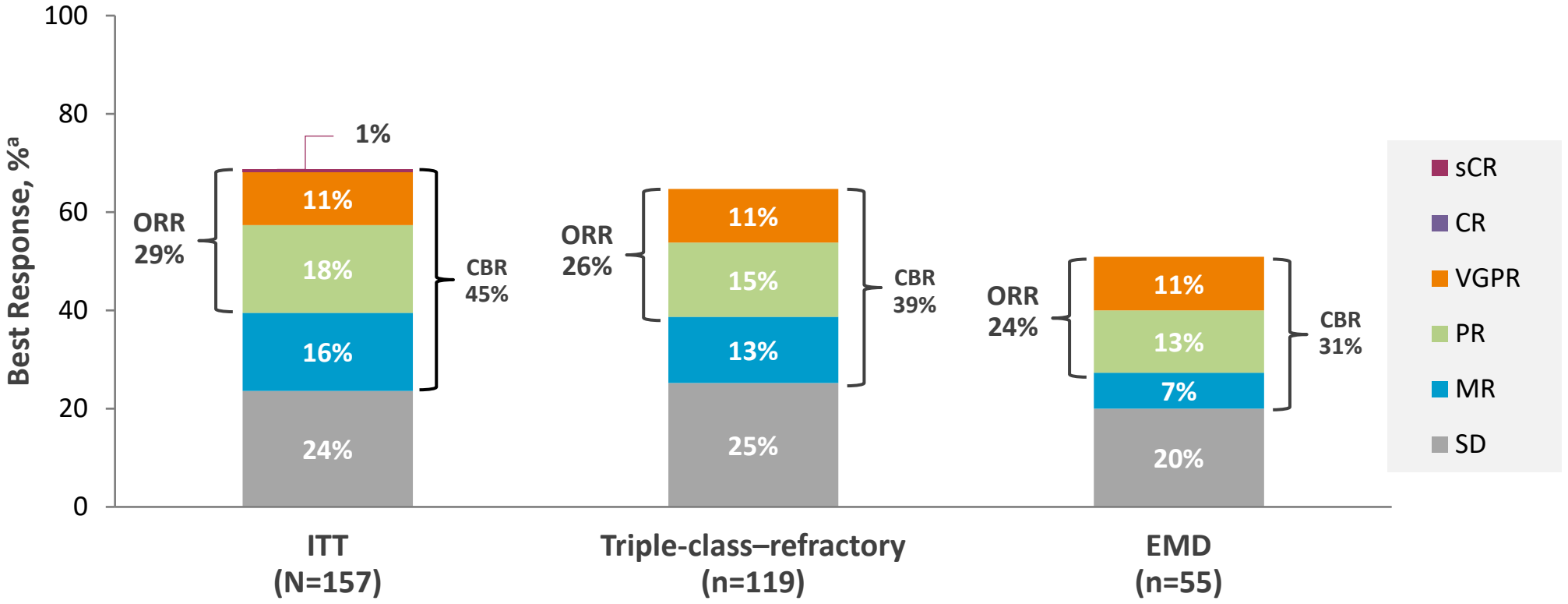
INTRODUCTION

Despite the introduction of novel therapies and regimens that have improved outcomes in multiple myeloma (MM),[1,2] almost all patients will relapse.[1,3] After relapse, treatment choice is usually determined by the class of and response to previous treatment and patient characteristics.[2,3] Although class switching is generally prioritized, this is becoming increasingly difficult, not least because novel agents are commonly administered in combination in earlier treatment lines, resulting in disease resistant to multiple drug classes as early as second-line therapy.[2,3] Outcomes are particularly poor for patients with high-risk cytogenetics, extramedullary disease, and MM resistant to multiple drug classes, including those with triple-class–refractory disease who represent groups with a high unmet need.[1,3,4] Furthermore, patients with relapsed and refractory multiple myeloma (RRMM) may have comorbidities because of age, disease symptoms, and cumulative toxicities stemming from previous therapies.[5,6] There is an urgent requirement for agents with novel mechanisms of action that are effective, safe, and tolerable and that maintain quality of life in patients with aggressive and resistant disease.

HORIZON STUDY – TOP LINE RESULTS

IN PATIENTS WITH HEAVILY PRETREATED RELAPSED AND REFRACTORY MM



In the ITT Population, the overall response rate was 29% with median duration of response at 5.5 months, median PFS was 4.2 months and median overall survival was 11.6 months. Grade ≥3 treatment emergent AEs occurred in 96% of patients, most commonly neutropenia (79%), thrombocytopenia (76%) and anemia (43%).

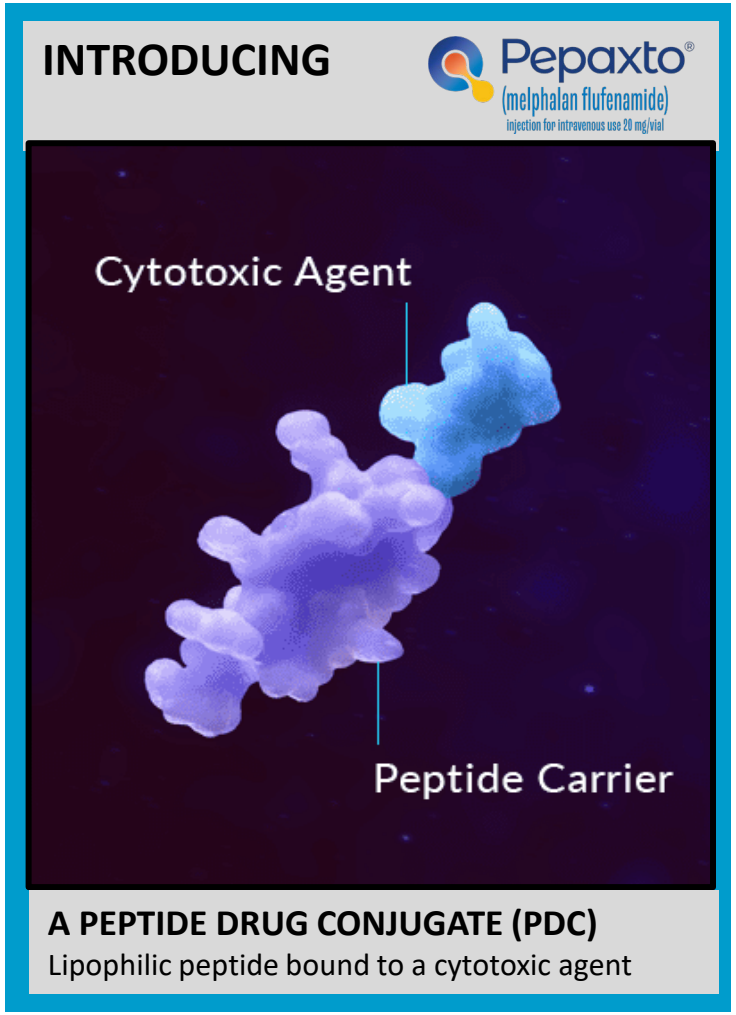
HORIZON data published in Journal of Clinical Oncology in December 2020

FDA GRANTS ACCELERATED APPROVAL IN RRMM

PEPAXTO - FIRST ANTI-CANCER PEPTIDE DRUG CONJUGATE

MECHANISM OF ACTION

Melphalan flufenamide is a **peptide conjugated alkylating drug.** Due to its **lipophilicity**, melphalan flufenamide is passively distributed into cells and thereafter **enzymatically hydrolyzed to melphalan**. Similar to other nitrogen mustard drugs, **cross-linking of DNA is involved** in the antitumor activity of melphalan flufenamide. In cellular assays, melphalan flufenamide **inhibited proliferation and induced apoptosis of hematopoietic and solid tumor cells**. Additionally, melphalan flufenamide showed synergistic cytotoxicity with dexamethasone in **melphalan resistant and non-resistant** multiple myeloma cell lines.



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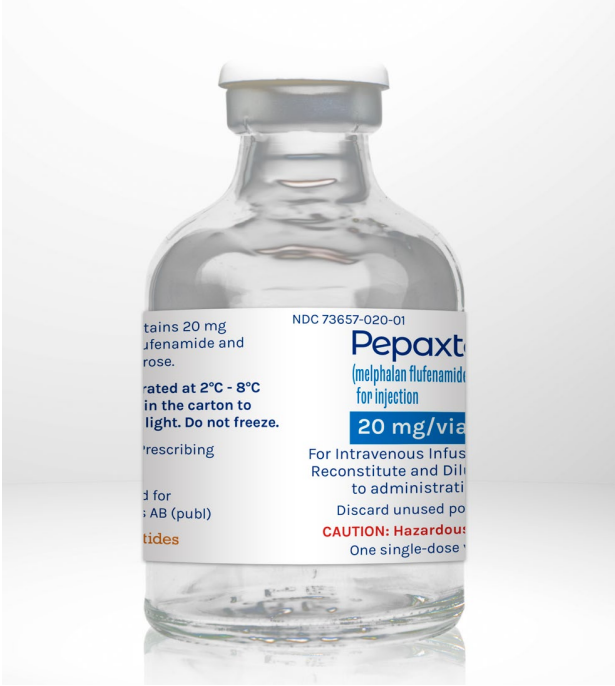
FDA GRANTS ACCELERATED APPROVAL IN RRMM

PEPAXTO OFFERS HOPE TO PATIENTS WITH HIGH UNMET MEDICAL NEED

- FDA approval based on a sub population of the HORIZON study (n=97) with high unmet medical need, defined in Table 5 of the label, of which 41% had extramedullary disease (EMD) and 75% had alkylator refractory disease
- Initial label targets patients with relapsed or refractory multiple myeloma, whose disease is refractory to at least one proteasome inhibitor, one immuno-modulatory agent, and one CD38-directed antibody, who have received at least four prior lines of therapy
- Commercial drug available to patients within 2 weeks



Pepaxto®
(melphalan flufenamide)
injection for intravenous use 20 mg/vial

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PEPAXTO DATA IN THE COMPETITIVE LANDSCAPE

TRIPLE CLASS REFRACTORY PATIENTS WITH >FOUR PRIOR LINES OF TREATMENT

	PEPAXTO **Oncopeptides** **US Approval, Feb 2021**	**Selinexor** **Karyopharm** **US approval, July 2019**	**Belantamab Mafodotin** **GSK** **US Approval, Aug 2020**
U.S label	Triple Class Refractory	Penta Refractory	Triple Class Exposed
Number of patients studied	97	122	95
Share of patients with EMD	41%	22%	20%*
Overall Response/Clinical Benefit Rate	24% / 37%	25% / 39%	31% / 36%*
mDOR / mPFS responders	4.2m / 8.7m	3.8m / 4.0m	11.0m/NR
Progression-free survival	3.8 months	3.7 months	2.9 months*
Overall survival	9.1 months	8.0 months	13.7 months*
Dose reduction, % of patients	27%	49%	29%
Gr3/4 bleeding events, % of patients	3.8%	3.0%	2.1%
Non-hematologic toxicity (grade 3/4) reported in >5% of patients	Pneumonia 11%**	Fatigue 25% Hyponatremia 20% Nausea 10% Pneumonia 9% Diarrhea 7% Sepsis 6% Hypokalemia 6% Mental status 6% General det. 6%	Keratopathy 44% Decreased Visual Acuity 28% Pneumonia 7% Pyrexia 6%

Source: FDA Label documents for PEPAXTO, Xpovio and Blenrep (items marked with '*' is data from DREAMM-2 as published in Lancet).

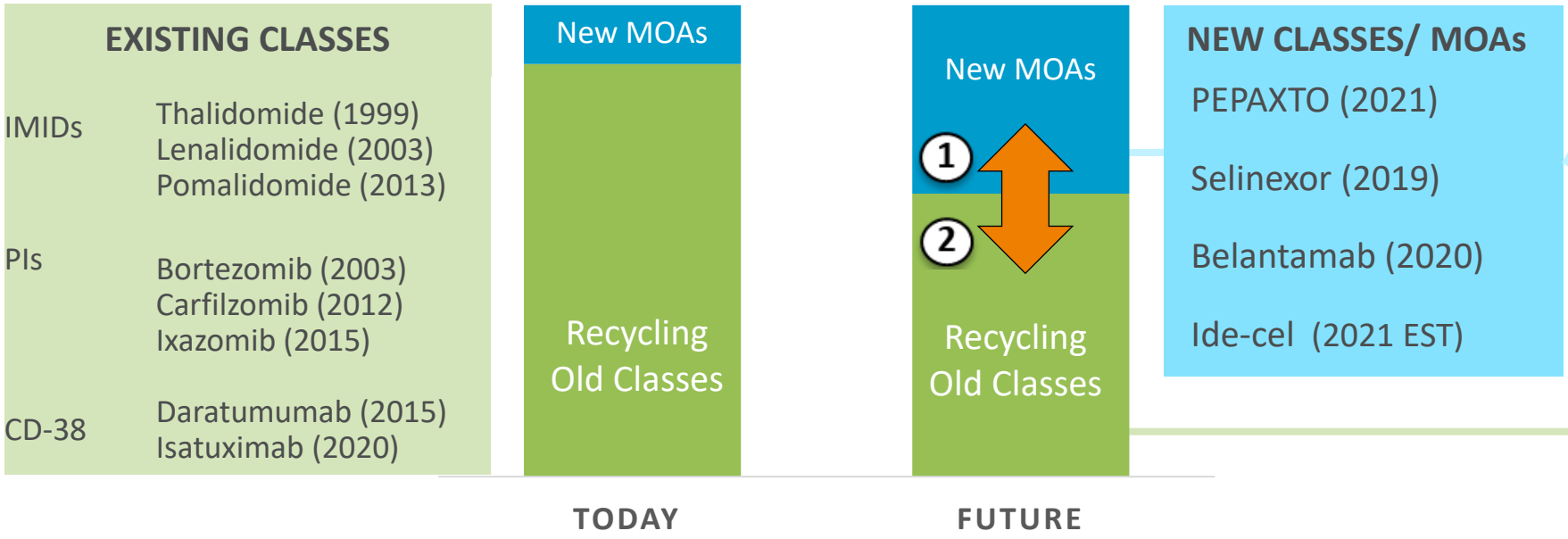
**Safety data based on 157 patients

TWO-PRONGED STRATEGIC APPROACH

BECOME TREATMENT OF CHOICE AND EXPAND MARKET

Driving change in today's RRMM treatment paradigm

Common Practice to "recycle" drugs within existing classes as patients progress



1. Become the treatment of choice for appropriate and indicated patients
2. Expand market is an opportunity for new MOAs No more "recycling" of failed drug classes

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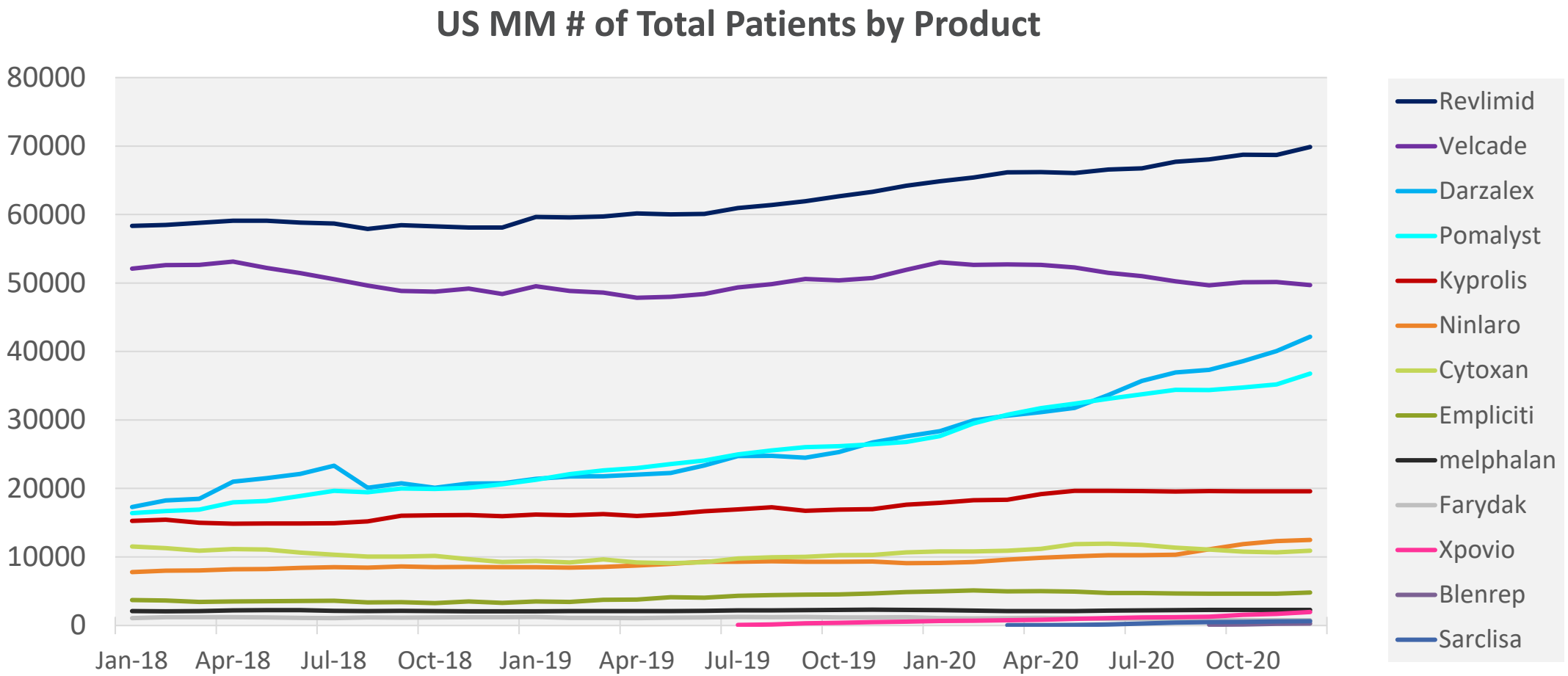
NEWER PRODUCTS ON TOP OF OLDER AS SURVIVAL IMPROVES

NEED OF NEW TREATMENT OPTIONS



Source: Intrinsiq MAT, December 2020

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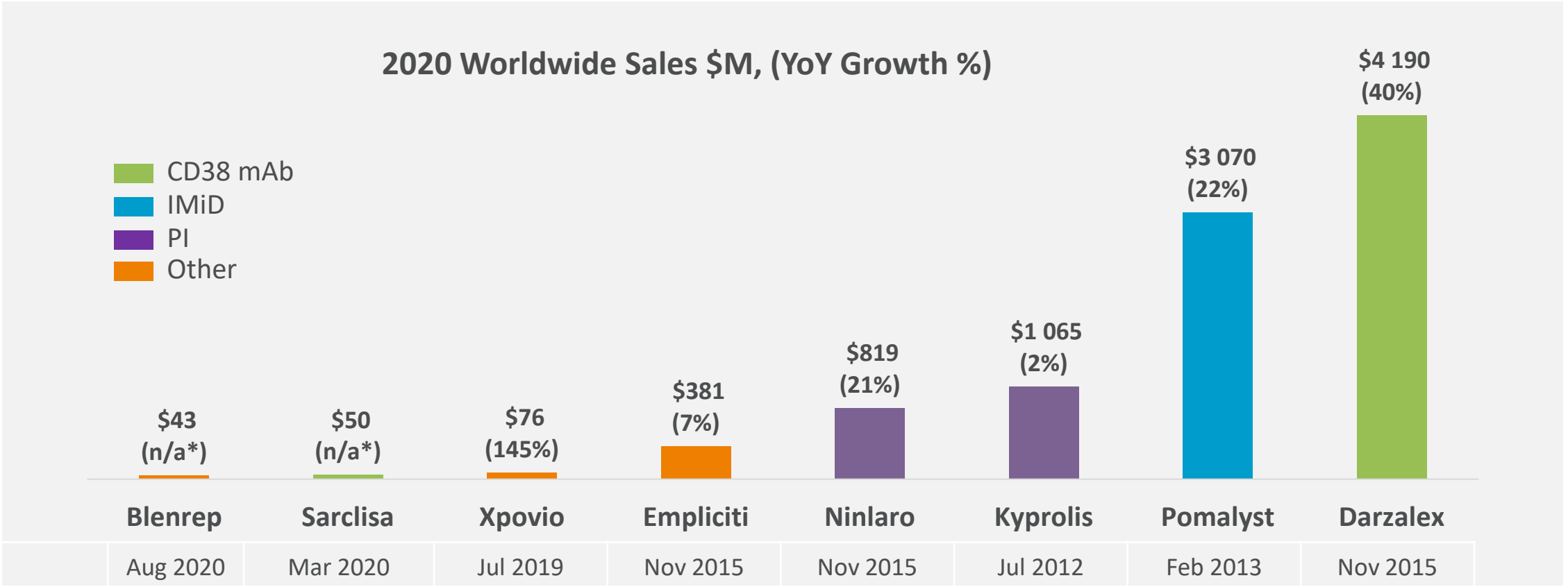
DRUGS WITH PEPAXTO'S PROFILE HAVE A SIGNIFICANT POTENTIAL



	Blenrep	Sarclisa	Xpovio	Empliciti	Ninlaro	Kyprolis	Pomalyst	Darzalex
Launch	Aug 2020	Mar 2020	Jul 2019	Nov 2015	Nov 2015	Jul 2012	Feb 2013	Nov 2015
First Year Sales, $M	$43*	$50*	$66	$153	$159	$189	$305	$592

*Newly approved products without full year sales. Source: EvaluatePharma, company earnings

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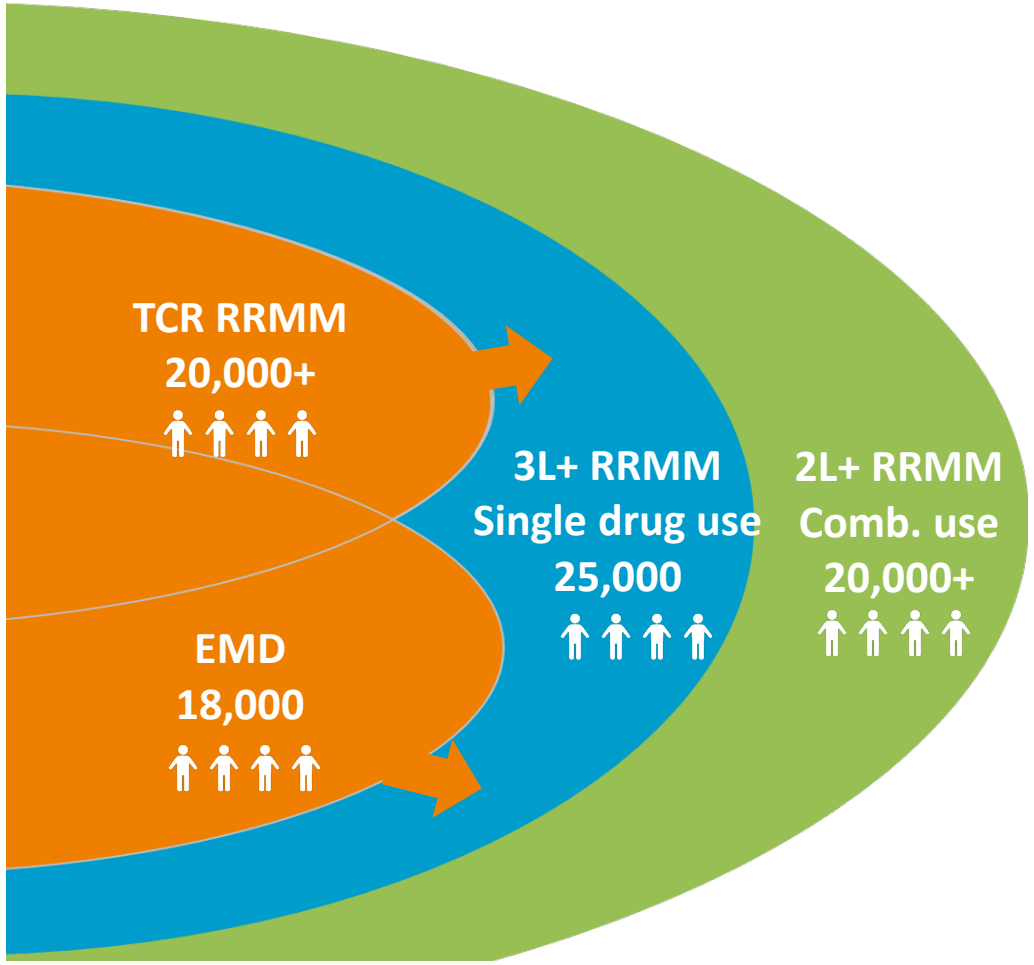
DEVELOPING PEPAXTO FOR RRMM PATIENTS

US MARKET – CURRENT GROSS PATIENT NUMBERS



Average duration of therapy

3-4 months | 6-9 months | 10-14 months

Clinical program supports label expansion

HORIZON
Approval in triple-class refractory (TCR) patients who have received at least 4L of treatment

OCEAN
Head-to-head study with pomalidomide may enable single agent 3L+ use

ANCHOR
LIGHTHOUSE
Combination with PI or anti-CD38 may enable 2L+ combination treatment

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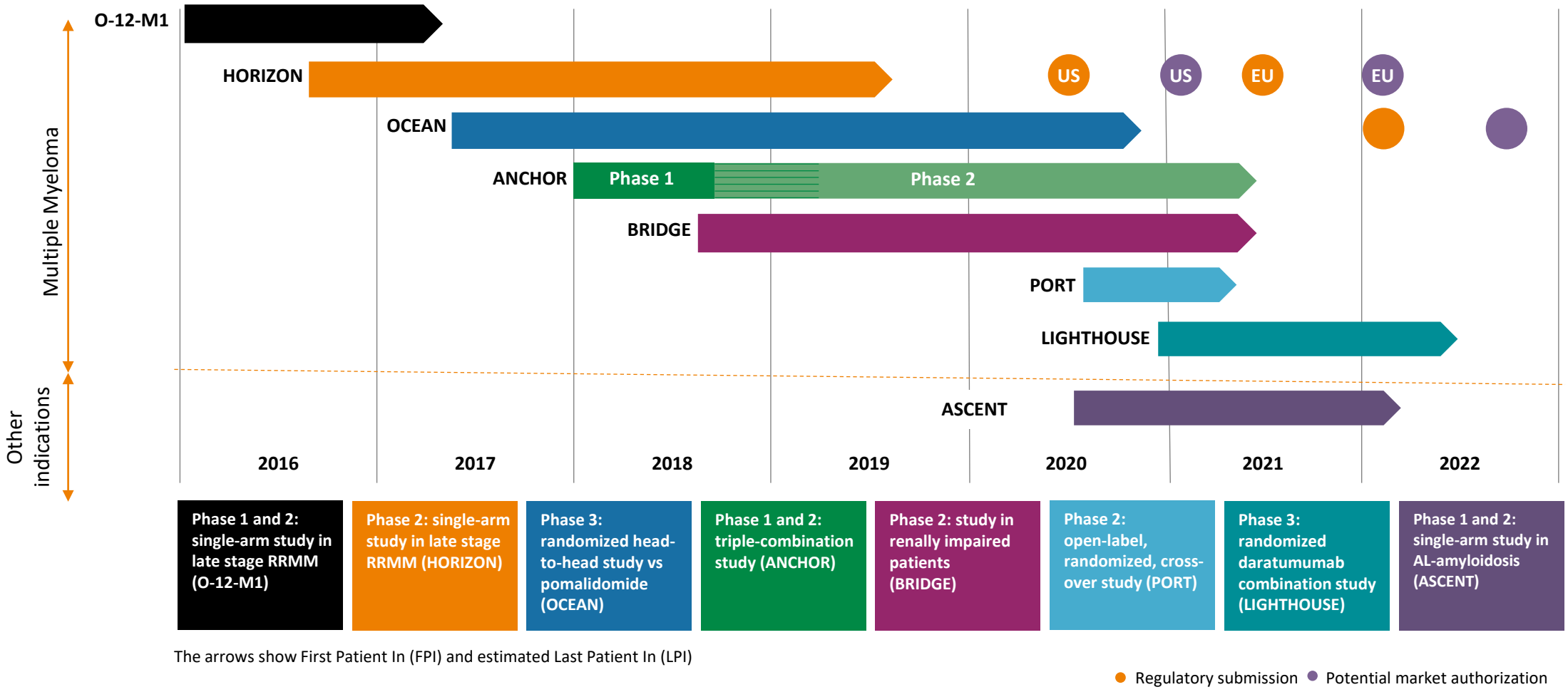
MELFLUFEN CLINICAL DEVELOPMENT PROGRAM

POTENTIAL TO PROVIDE DATA IN DIFFERENT PATIENT POPULATIONS



The arrows show First Patient In (FPI) and estimated Last Patient In (LPI)

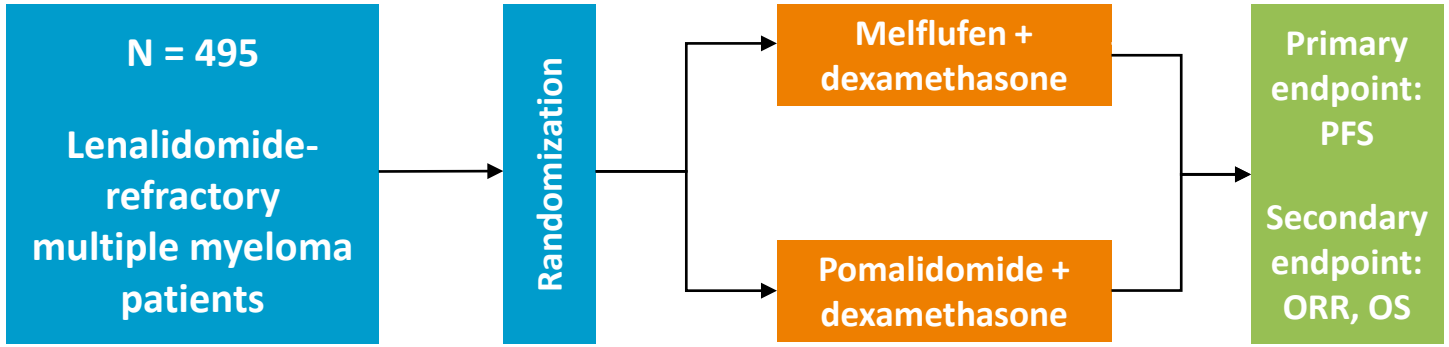
● Regulatory submission ● Potential market authorization

LABEL EXPANSION OPPORTUNITY WITH PHASE 3 OCEAN STUDY

CONFIRMATORY STUDY – TOPLINE RESULTS Q2 2021

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



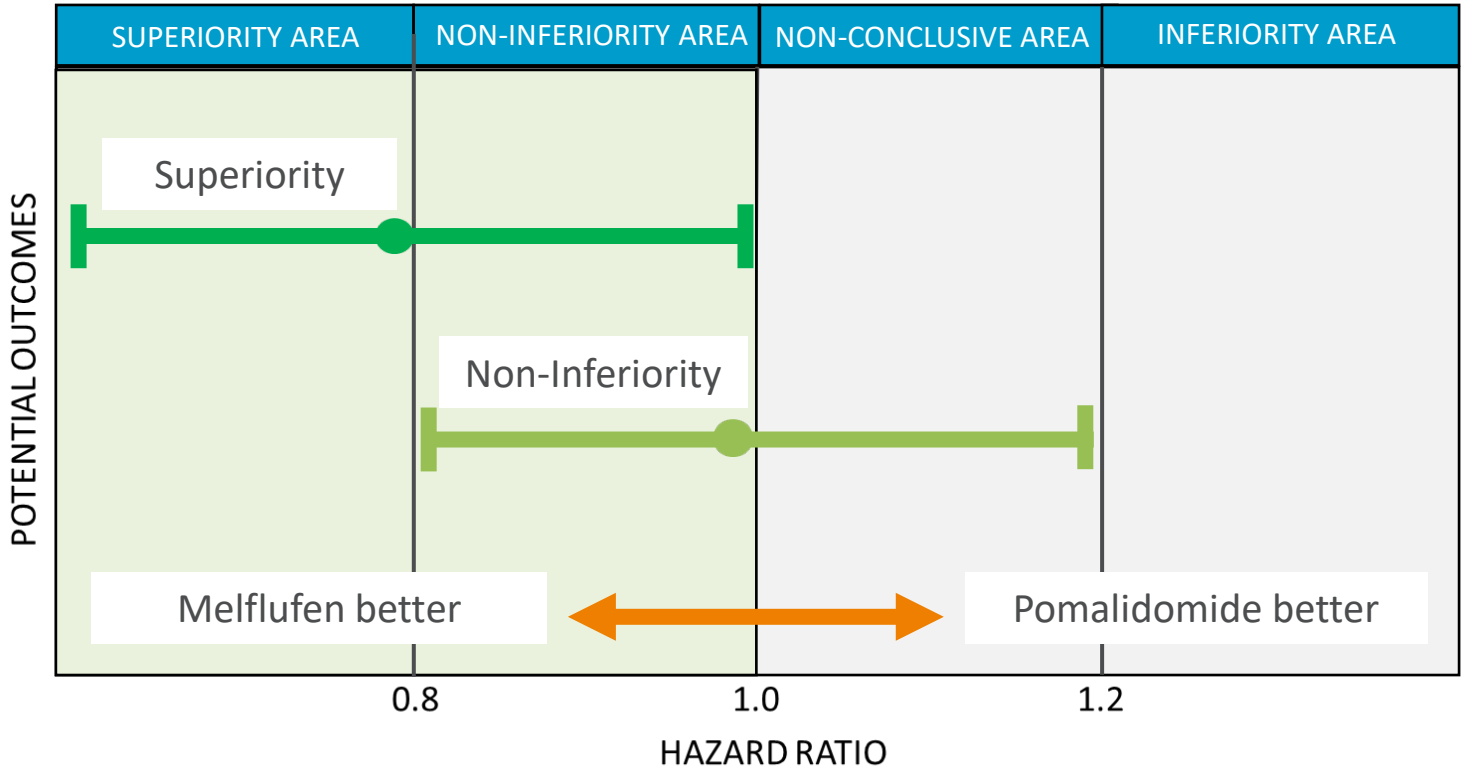
RRMM data from pomalidomide FDA label and O-12-M1 study

Treatment	ORR	CBR	Median PFS	Median DOR	Median OS
Melflufen + Dexamethasone	31%	49%	5.7 months	8.8 months	20.7 months
Pomalidomide+ Dexamethasone	24%	NR	3.6 months	7.0 months	12.4 months

TWO WAYS TO MEET THE PRIMARY ENDPOINT IN OCEAN

HEAD-TO-HEAD STUDY WITH POMALIDOMIDE – TOPLINE RESULTS Q2 2021

- OCEAN meets its primary endpoint with a Superiority or Non-inferiority result



Regulatory implications

OUTCOME	FDA	EMA
Primary endpoint met - **Superiority**	✓	✓
Primary endpoint met – **Non-inferiority**	Data driven	✓
Primary endpoint **not** met	✗	✗

LIGHTHOUSE STUDY - BASED ON POSITIVE ANCHOR DATA

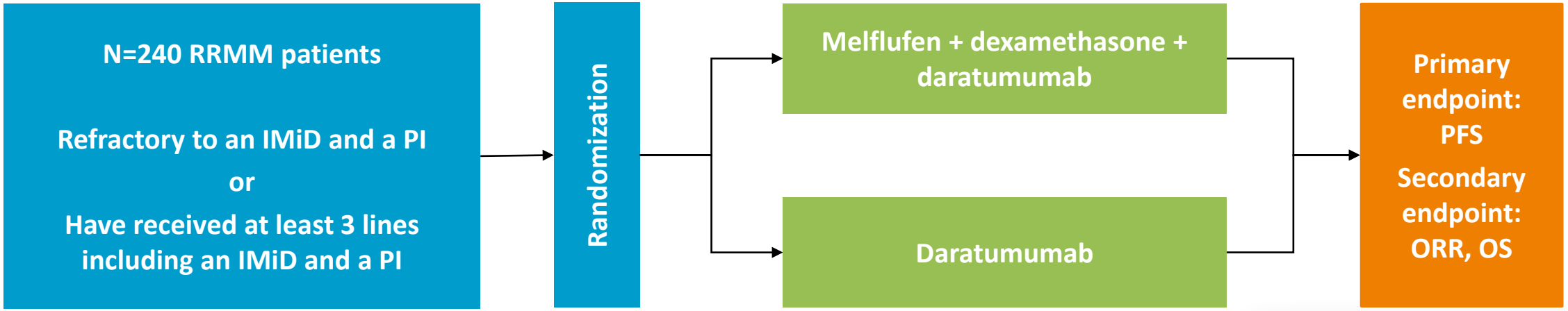
CONFIRMATORY PHASE 3 STUDY – INITIATED IN DECEMBER 2020

Phase 3 study with melflufen in multiple myeloma

- Melflufen + daratumumab vs daratumumab randomized 1:1
- Subcutaneous version of daratumumab
- Based on promising melflufen + daratumumab data from ANCHOR (ORR 73%, m PFS 12.9 months)

Objectives

- Expand market potential – expand label for melflufen in combination with daratumumab



PLANNED FUTURE STUDIES

Expanding in myeloma

EXTRAMEDULLARY DISEASE

Combination bortezomib-melflufen-dexamethasone in soft-tissue extramedullary disease (EMD)
Building on positive HORIZON data in EMD

Phase 2 study LANTERN
FPI expected H2 2021

NOVEL COMBINATIONS

Combination study with BiTe or CAR-T – to enable label expansion in combination treatments
Phase 2/3 study

In planning – FPI 2022

Expanding in new indications

ACUTE MYELOID LEUKEMIA (AML)

High unmet medical need – limited survival – OS less than a year

Phase 1/2 study in relapsed patients

FPI expected H2 2021/Q1 2022

NHL: RELAPSED DIFFUSE LARGE B-CELL LYMPHOMA (DLBCL)

High unmet medical need – limited survival

Phase 1/2 study in relapsed high-risk patients
FPI expected H2 2021

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NEWS FLOW
VALUE DRIVERS AND MAJOR MILESTONES

Q4 2020	Q1 2021	Q2 2021	H2 2021	H1 2022
Expanded Access Program (US) opened	Accelerated approval in US	Top-line results OCEAN	Results BRIDGE	Potential conditional approval in EU
Intent to file for EU conditional approval	Commercial launch in the US	Application for CMA to EMA	Results PORT	Final results ANCHOR
Loan agreement with EIB for € 40 M	Pepaxto® (melphalan flufenamide) injection for intravenous use 20 mg/vial	FPI COAST (OPD5)	LPI ANCHOR	LPI LIGHTHOUSE
IND filing OPD5		LPI PORT	LPI BRIDGE	Potential sNDA submission OCEAN
ASH abstract including ANCHOR data		EHA data update	LPI ASCENT	Extension of EU indication on OCEAN
Virtual CMD			FPI LANTERN (EMD)	
ANCHOR presentation at ASH			FPI in “signal seeking” melflufen trial(s)	
HORIZON publication Journal Clin Onc				
First patient in LIGHTHOUSE				

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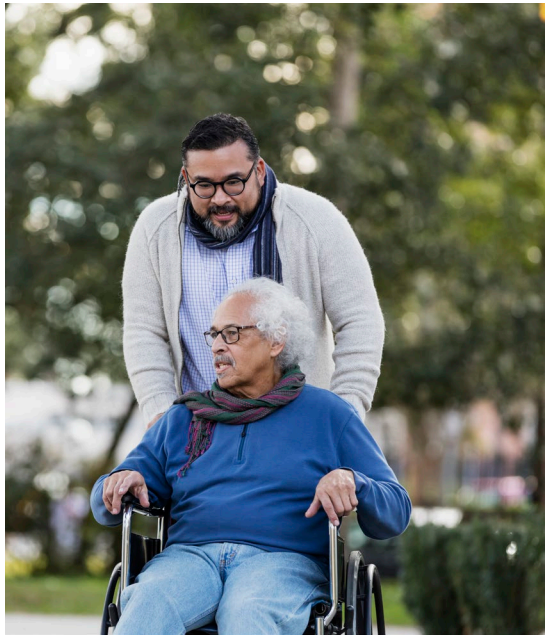
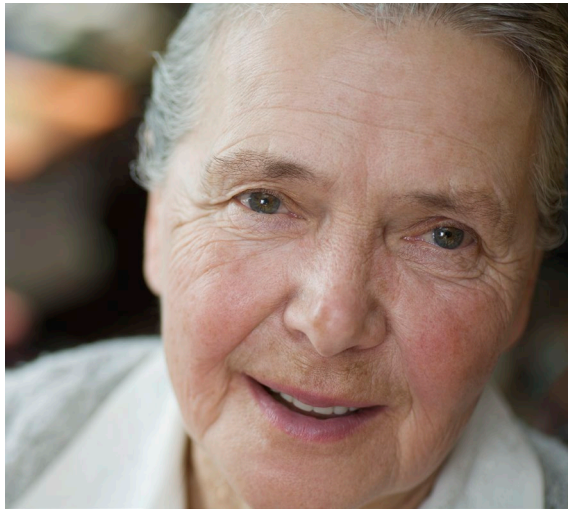
Pepaxto®
(melphalan flufenamide)
injection for intravenous use 20 mg/vial

ADDRESSING A GROWING UNMET MEDICAL NEED

SCIENCE · TRUST · COURAGE · COLLABORATION · PASSION





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