

# **Oncopeptides**

**SEB Annual Pharma & Biotech Seminar  
January 22, 2020**

**Jakob Lindberg, CEO**



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# Oncopeptides at a glance

## Develops targeted cancer treatments

- Proprietary peptide-conjugated compounds
- Lead compound Melflufen a peptide-conjugated alkylator targeting Multiple Myeloma

## Initial focus on Multiple Myeloma

- Significant market opportunity in orphan indication
- Melflufen Phase 2 study, O-12-M1, showed the best MM survival data to date

## Application process initiated for accelerated approval in the US

- Target to submit in H1-20 based on ongoing phase 2 study HORIZON
- Triple-class refractory MM

## Phase 3 expected to be fully enrolled in Q1 2020

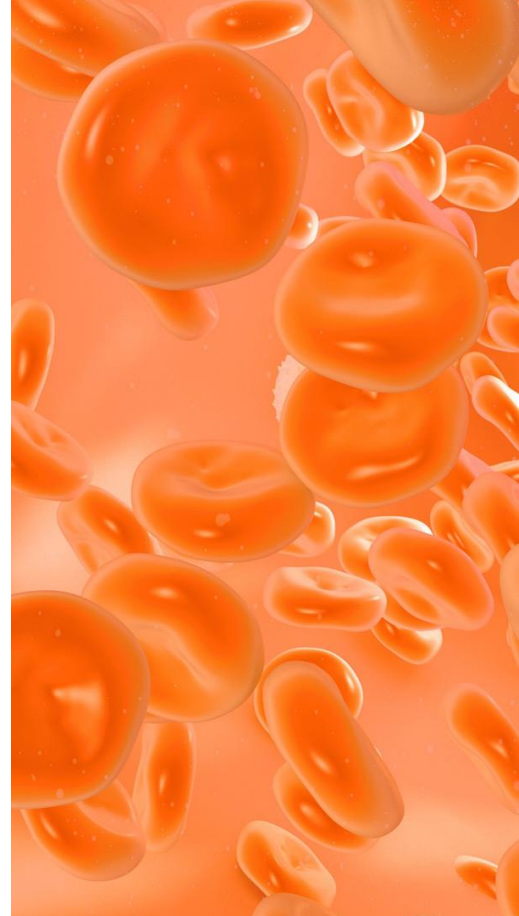
- Approximately 450 patients at 140 sites
- Two additional supporting trials ongoing. Additional phase 3 called LIGHTHOUSE will start early 2020

## New indications and NCEs in development

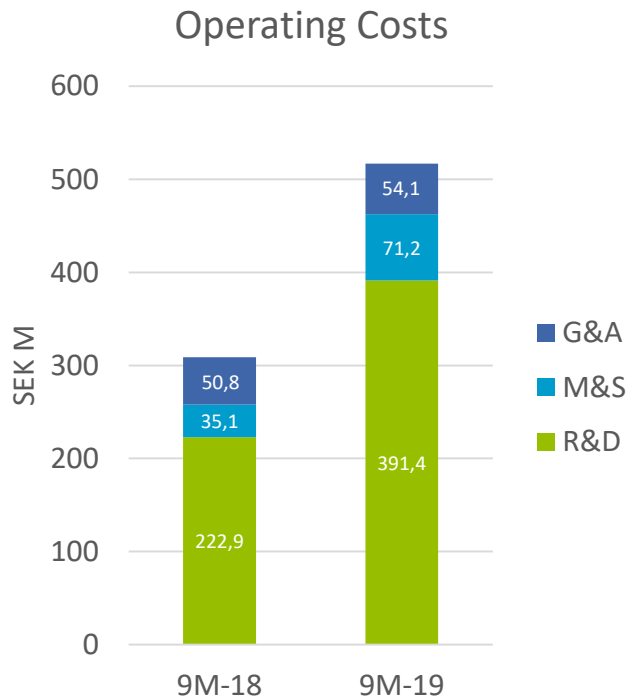
- A Phase 1/2 study addressing AL amyloidosis to start shortly

## Listed on NASDAQ Stockholm, strong financial position

- Market cap: SEK 7,4 B (\$ 780 M)
- Cash position: SEK 1,122 M (\$ 118 M) as of September 30



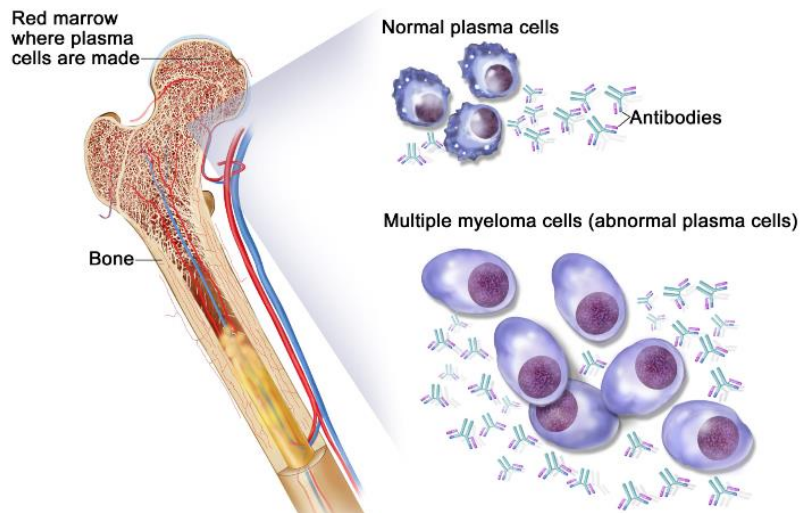
# Financial results for the period Jan – Sep 2019



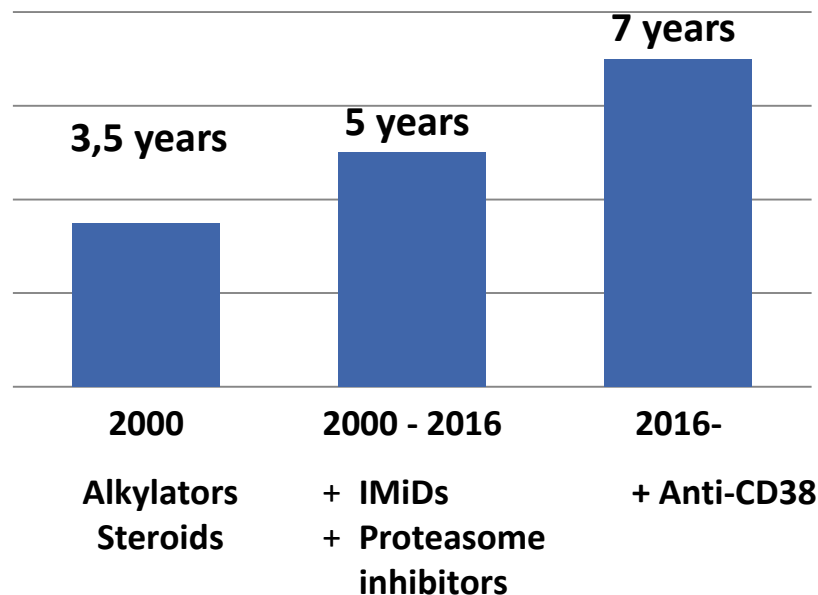
- Operating loss increased to SEK 495.1 M (loss:299.1)
  - R&D increase primarily due to increase in Clinical & drug supply: SEK 318.3 M (189.9)
    - OCEAN costs SEK 174.2 M (100.7)
    - HORIZON costs SEK 41.2 M (18.1)
    - ANCHOR costs SEK 30.8 M (16.8)
  - Build-up of commercial and medical relations explains increase in M&S costs
- Operating costs include non-cash costs related to incentive programs
  - SEK 24.1 M (61.7) for the first nine months
- Cash flow from operating activities neg. SEK 473.6 M (neg. 224.9)
- Cash position was SEK 1 122.3 M (488.9) as of Sep 30, 2019
  - Directed share issue raised SEK 514.8 M after issue costs in January 2019
  - Second share issue raising SEK 682.9 M was completed in July

# Multiple Myeloma is a hematological cancer without cure

## Myeloma – Uncontrolled plasma cell proliferation



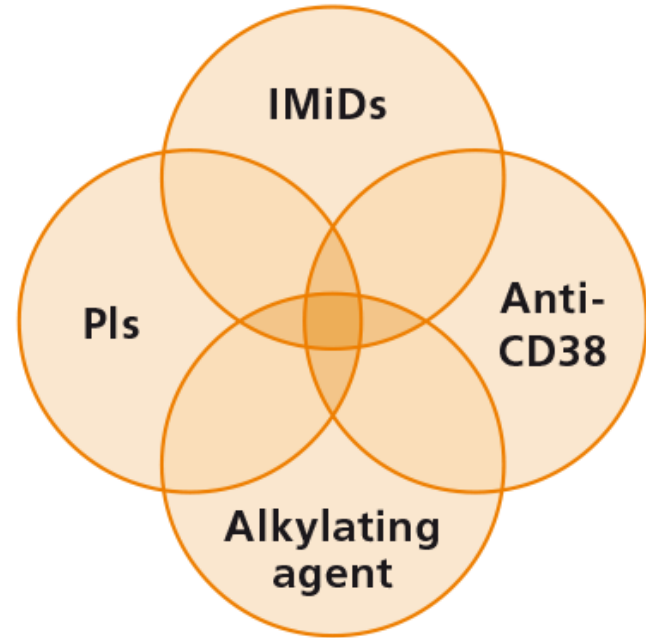
## Median Survival increasing with more available treatment options



Source: IntrinsicQ and Kantar Health.

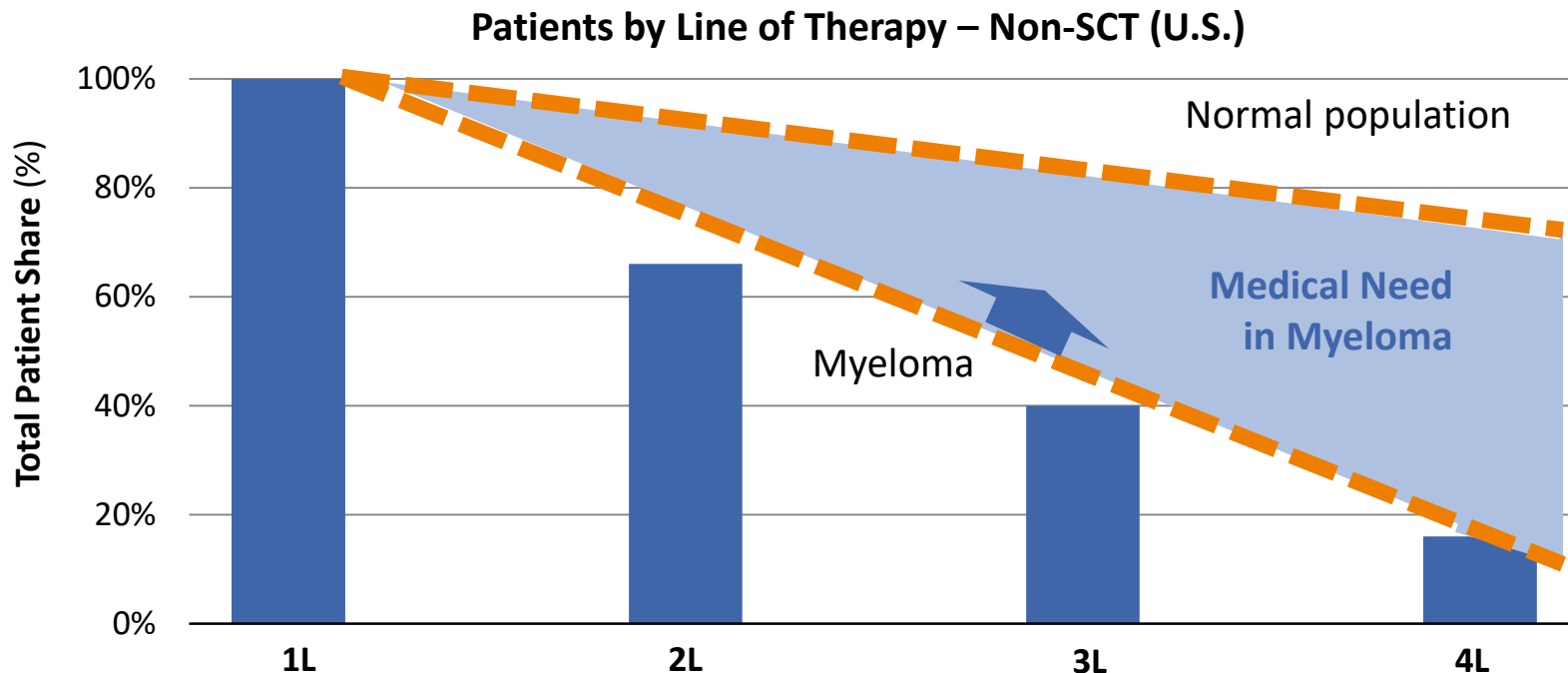
# Significant medical needs remain

- Four treatment modalities used with inevitable resistance development
- Currently, the majority of patients have been treated with all four modalities after 2-3 lines of therapy with limited treatment options left
- Frequent co-morbidities further compounding the problem with limited treatment options



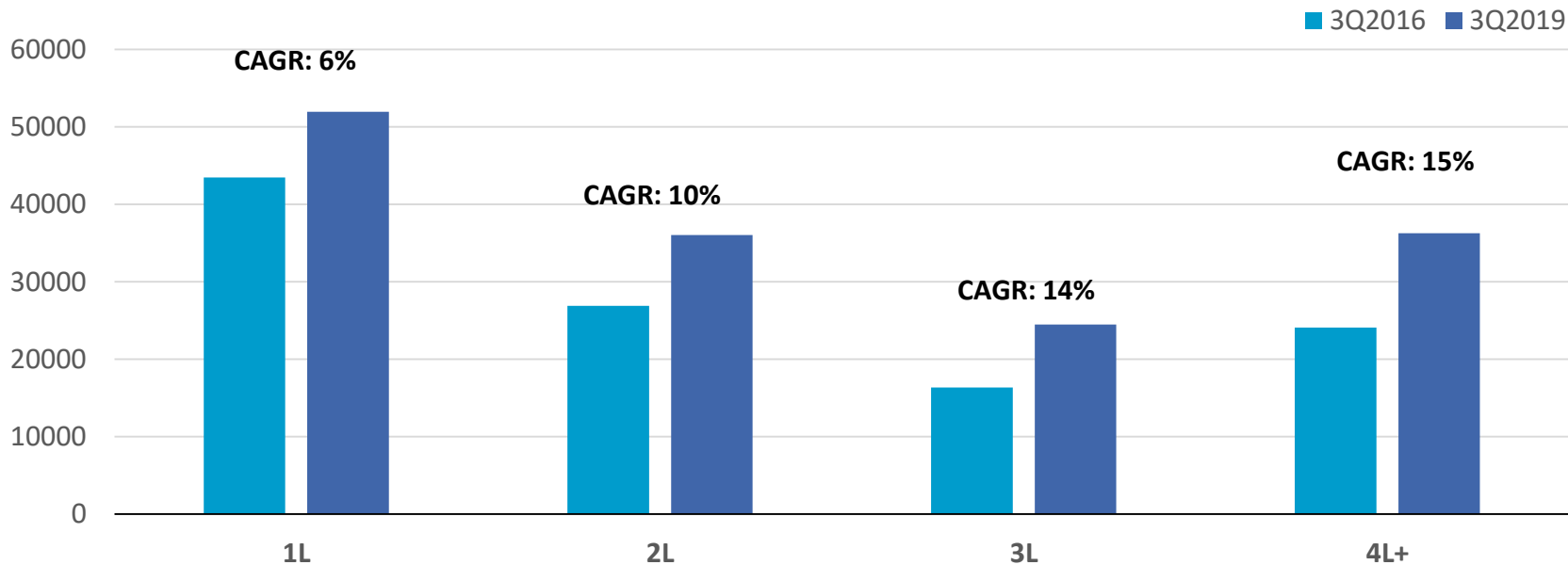
# We are still far from making myeloma a chronic disease

- Later line patient population growing with significant need for new treatments



# Improved outcomes lead to fast growth in number of treated patients in later lines of therapy

Projected US multiple myeloma patients by line of therapy



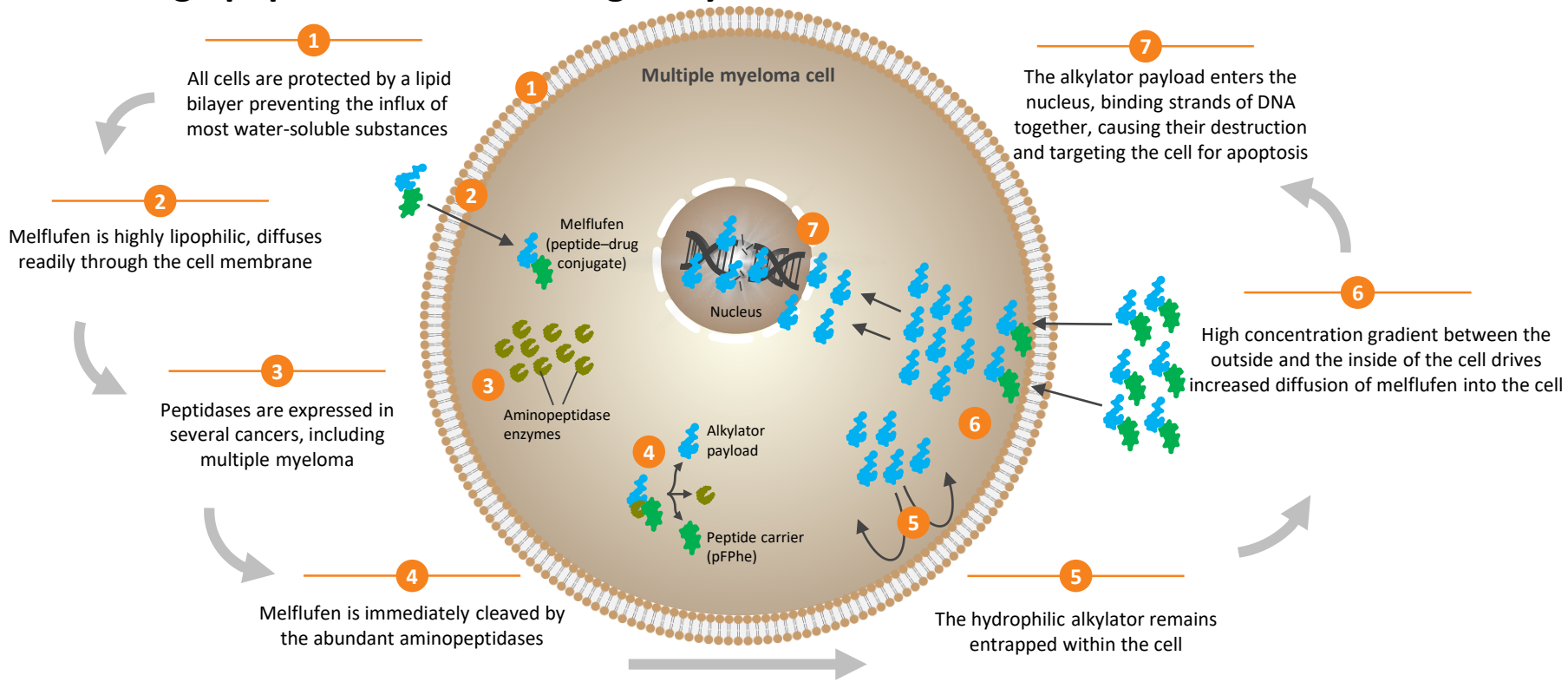
Source: Intrinsic MAT 3Q2019

Note: 3-yr annual growth rate for 3Q2016-3Q2019

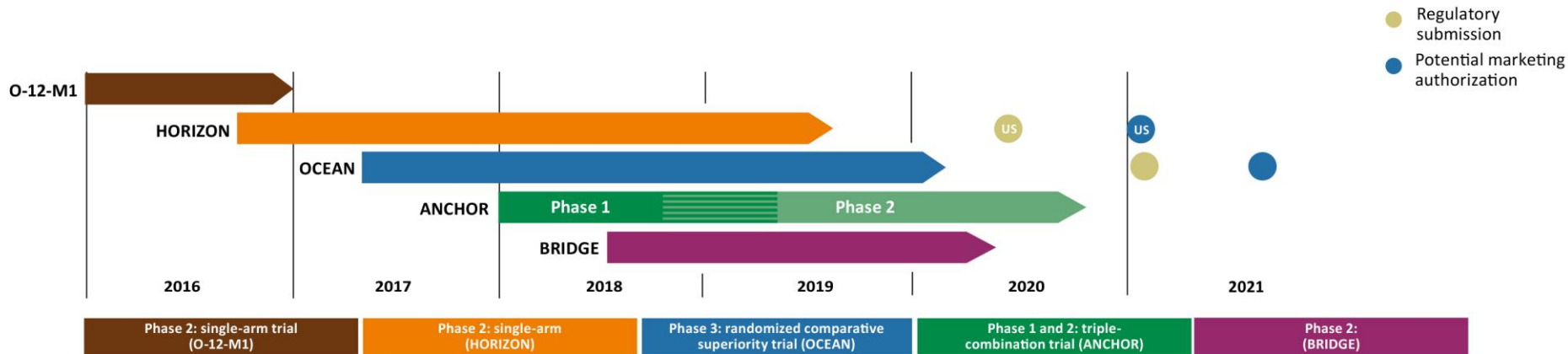


# Melflufen is a novel peptide-drug conjugate

- Uses high peptidase levels to target myeloma cells



# Overview of our present clinical development program in multiple myeloma



## O-12-M1

Show single-agent activity in RRMM

## HORIZON

Show single-agent activity in RRMM

## OCEAN

Show single-agent Superiority over SoC backbone in RRMM (pomalidomide)

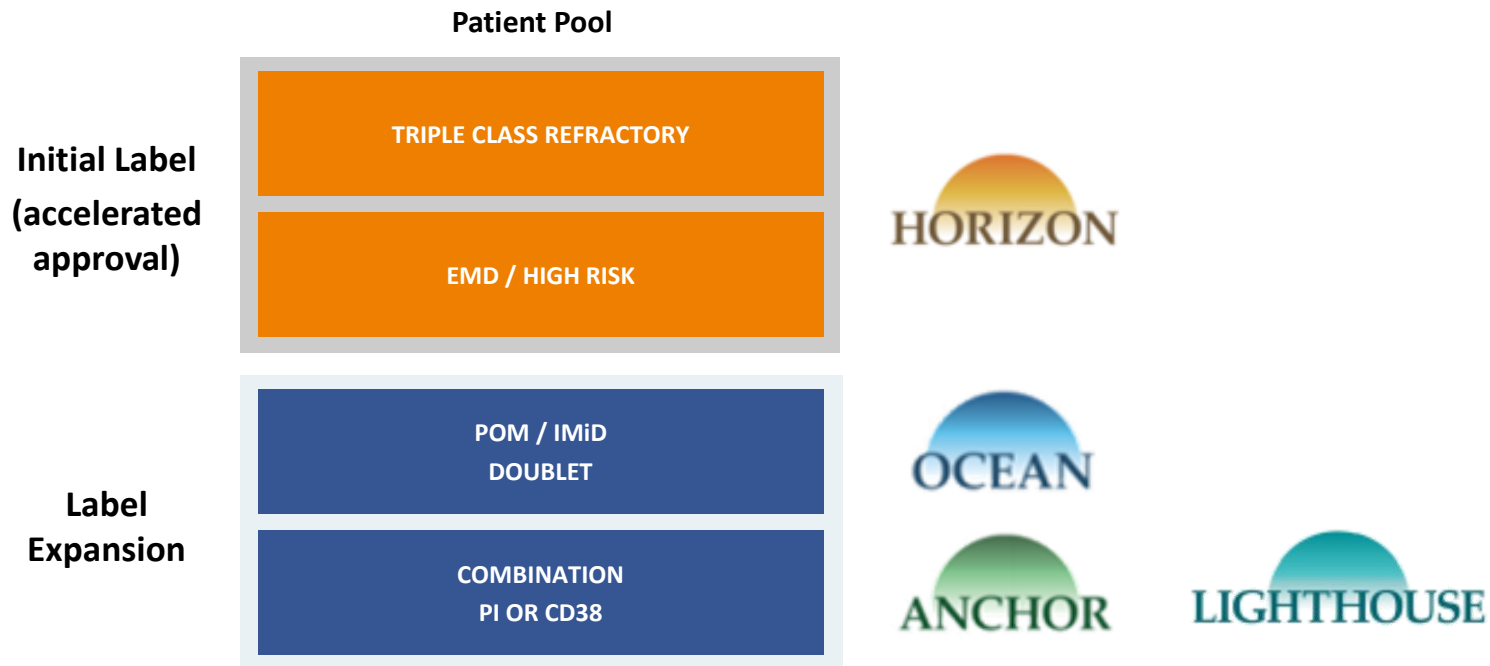
## ANCHOR

Show combination synergy and tolerability with daratumumab and bortezomib

## BRIDGE

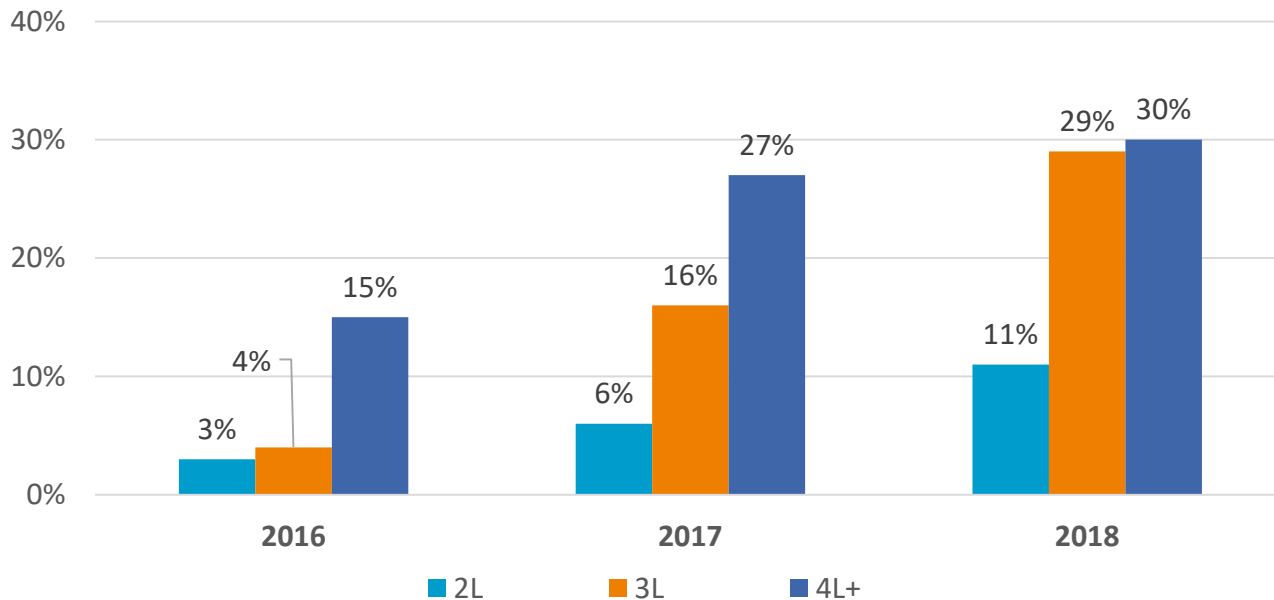
Show that melflufen can be used in patients with renal impairment

# Label journey with current development program in myeloma



# Initial indication of triple-class refractory disease is a significant and growing unmet medical need in myeloma

Triple-class refractory % patients after each line of therapy



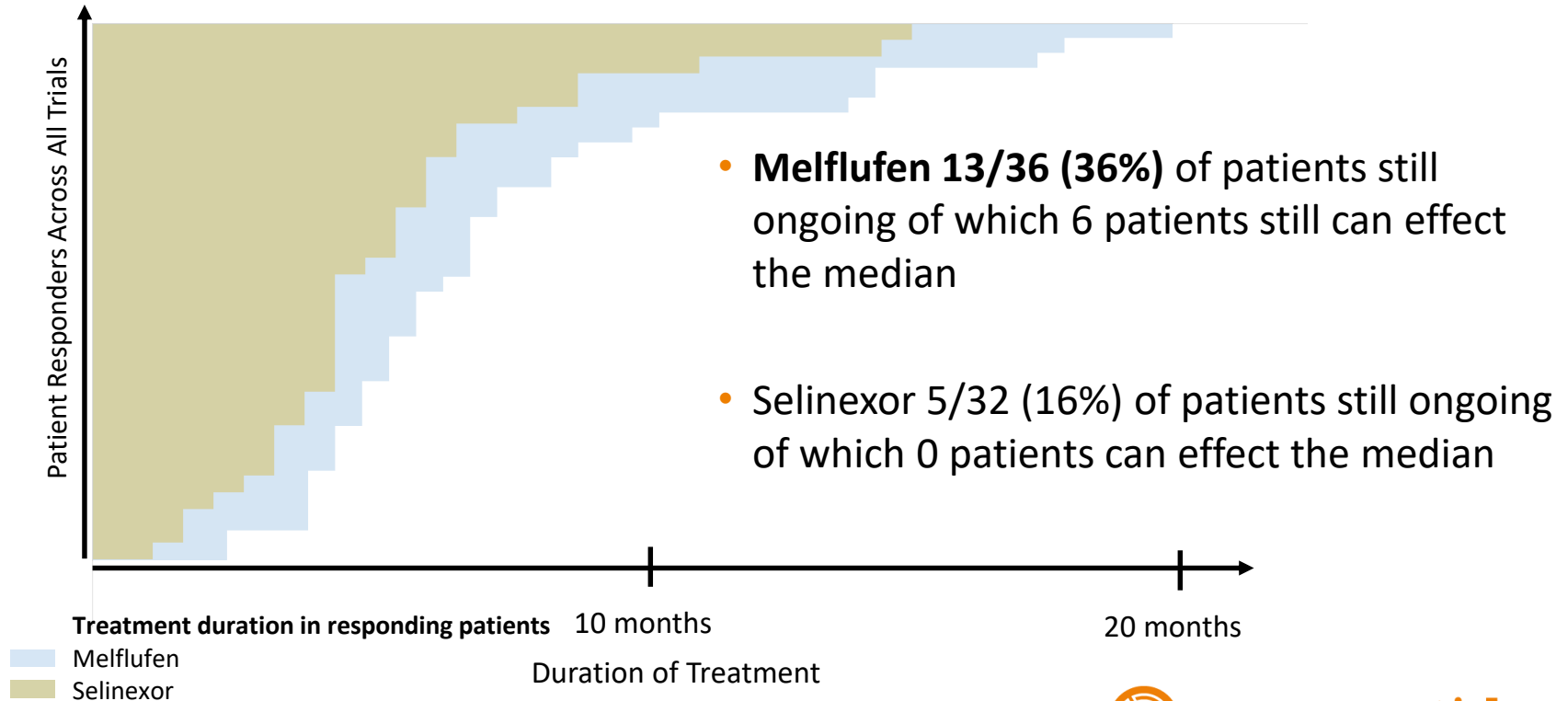
Estimated  
>20,000  
Triple-class  
refractory  
patients in the  
US and  
growing

# Melflufen triple-class RRMM data is highly competitive

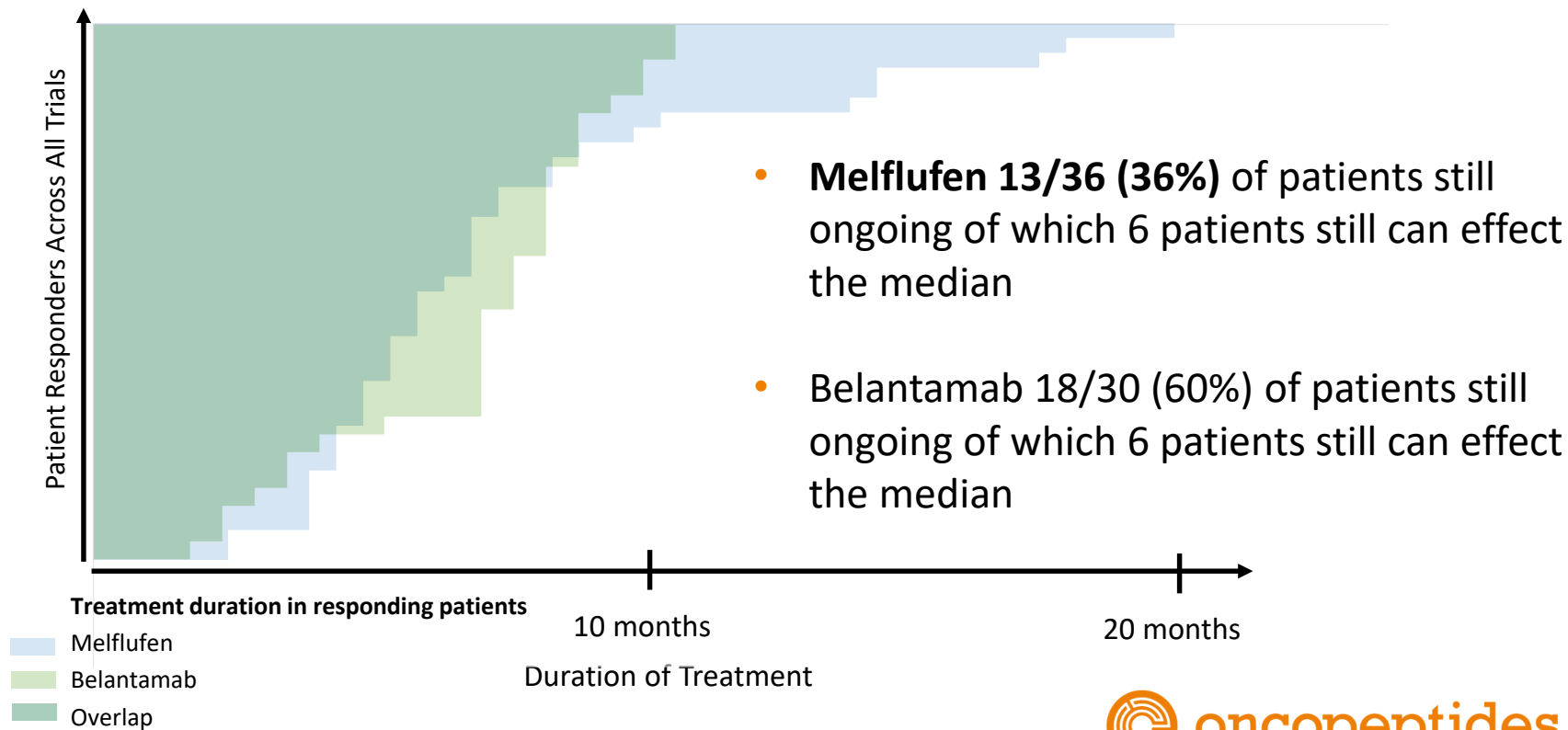
– Duration of treatment and QoL are main drivers of use in later lines of therapy

	Melflufen	Selinexor	Belantamab
ORR/CBR	24%/37%	25%/39%	31%/34%
mDOR	7.5 months	4.4 months	NR (≈7-8months)
mPFS	4.0 months	3.7 months	2.9 months
mOS	11.3 months	8.0 months	NR (≈10months)
%EMD	34%	22%	23%
SAE rate	51%	58%	36% (excl. ocular tox.)
<b>Non-hematologic toxicity (grade 3/4) reported in &gt;5% of patients</b>	Pneumonia 8.4%	Fatigue 25.2% Hyponatremia 20.3% Nausea 9.8% Pneumonia 8.9% Diarrhea 7.3% Sepsis 5.7% Hypokalemia 5.7% Mental status 5.7% General det. 5.7%	Keratopathy/ 27.4% Blurred vision Hypercalcaemia 7.4% Pneumonia/ 6.3% Lung infections

# Duration of treatment: Comparison between melflufen and selinexor



# Duration of treatment: Comparison between melflufen and belantamab



Source: The Lancet 2019, Melflufen Data Presentation, ASH 2019

# Requirements for success in Relapsed Refractory Multiple Myeloma

## MUST HAVE CHARACTERISTICS

Single agent +/- steroid activity in multi-refractory patients of >20% Overall Response Rate

Single agent +/- steroid approval in refractory patients

Efficacy synergy in combination with other main myeloma drugs with good tolerability

No major quality of life/ tolerability issues

No co-morbidity limitations

## NICE TO HAVE CHARACTERISTICS

Easy administration schedule

Proven single agent activity

 Pomalyst<sup>®</sup>

 DARZALEX<sup>®</sup>

Comorbidity or tolerability limitations

 Kyprolis<sup>™</sup>

 FARYDAK<sup>®</sup>  
(panobinostat) capsules  
10mg / 15mg / 20mg

Limited to no single agent data

 NINLARO<sup>®</sup>

 Empliciti<sup>™</sup>  
(elotuzumab)



# Development program for Melflufen is designed to support its potential as a new agent after IMiD and PI failure

## MUST HAVE CHARACTERISTICS

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## NICE TO HAVE CHARACTERISTICS

Easy administration schedule

## MELFLUFEN

O-12-M1 showed an ORR of 31% and HORIZON an ORR of 29% in multi-refractory patients

OCEAN head to head study vs. Pomalyst/dex is designed for approval

ANCHOR shows excellent synergy and good tolerability with daratumumab and bortezomib (early data)

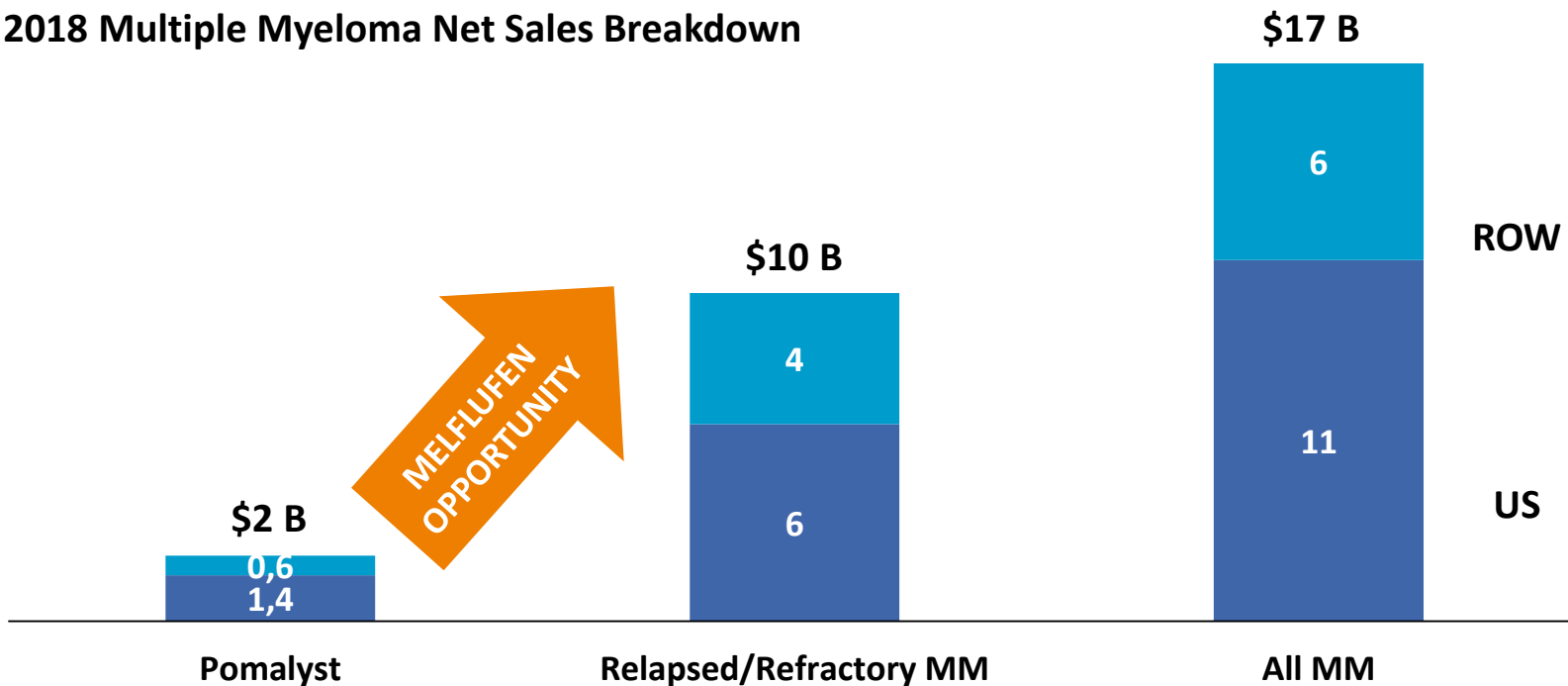
Good QoL with almost no non-hematological AEs

No co-morbidity or drug-drug interactions limitations

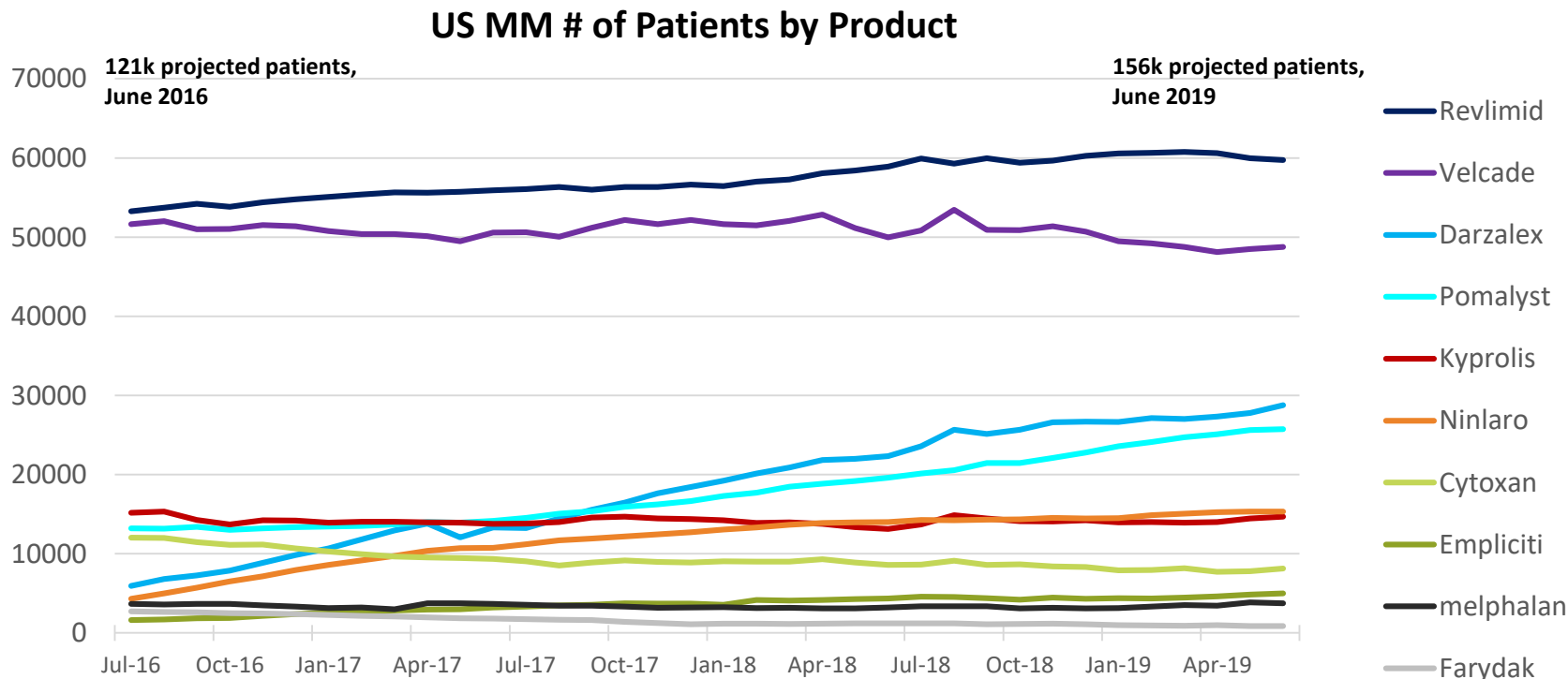
One 30-minute infusion every 28 days

# Melflufen opportunity in Relapsed Refractory Multiple Myeloma

– 2018 Multiple Myeloma Net Sales Breakdown



# Newer products used in addition to, not in place of, older products as survival improves



Source: Intrinsic MAT, June 2019

# Recent highlights

## Clinical programs progressing

- HORIZON fully enrolled in September
- ANCHOR meflufen + daratumumab arm fully enrolled in September
- BRIDGE trial expanded to include patients with severe renal impairment
- OCEAN on track to recruit last patient in Q1-20

## Promising clinical data presented at ASH and IMW

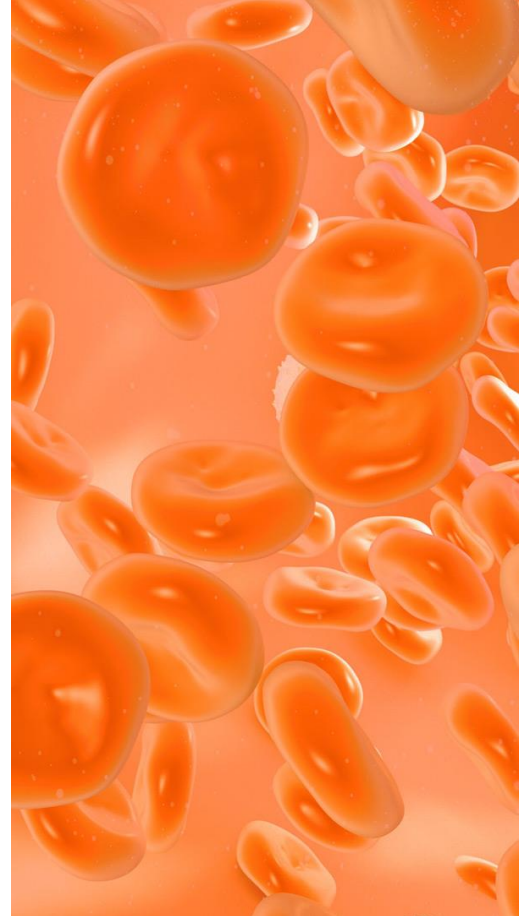
- ORR of 29% in HORIZON, 24% in triple-class refractory myeloma patients
- Progression-free survival of 14.3 months for melflufen in combination with daratumumab in RRMM (ANCHOR study)

## NDA submission process on track with submission planned during first half of 2020

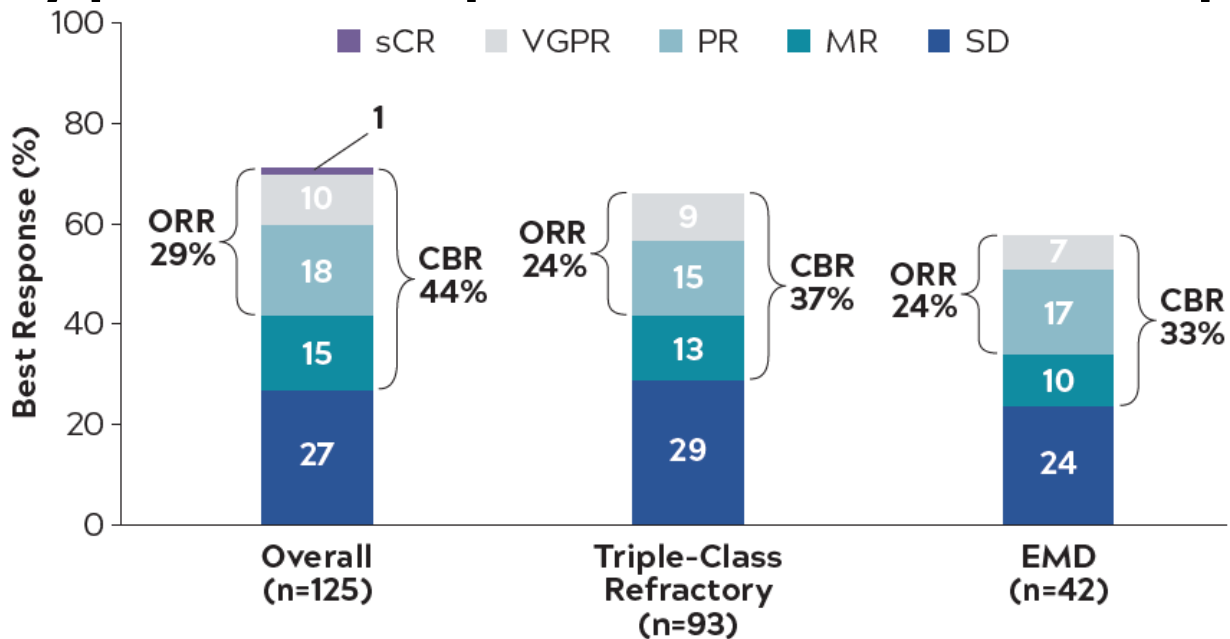
- Application for accelerated approval in triple class refractory patients based on HORIZON data
- Pre-NDA meeting recently held with the FDA confirming plans

## Key staff members recruited

- Klaas Bakker, MD, PhD, started as Chief Medical Officer in early November
- Joe Horvat started as President North America in early December



# Promising overall response rates in both triple-class refractory patients and patients with EMD at relapse

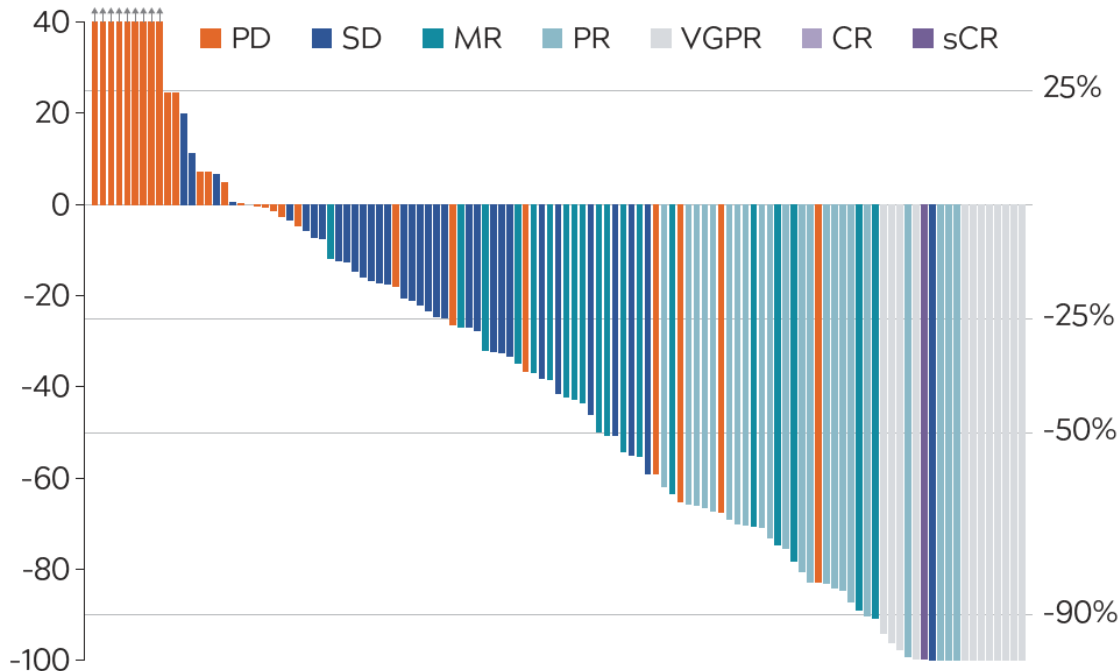


<sup>a</sup>Response was investigator assessed.

CBR, clinical benefit rate; EMD, extramedullary disease; IMWG, International Myeloma Working Group; MR, minimal response; ORR, overall response rate; PR, partial response; sCR, stringent complete response; SD, stable disease; VGPR, very good partial response.

Source: Mateos MV, et al. ASH 2019. #1883

# Disease was stabilized in 83% of patients<sup>a</sup>



- Overall, 83% of the patients had a reduction of M-protein despite all patients having progressing disease at study entry

<sup>a</sup>In total, 10 patients had missing M-protein data.

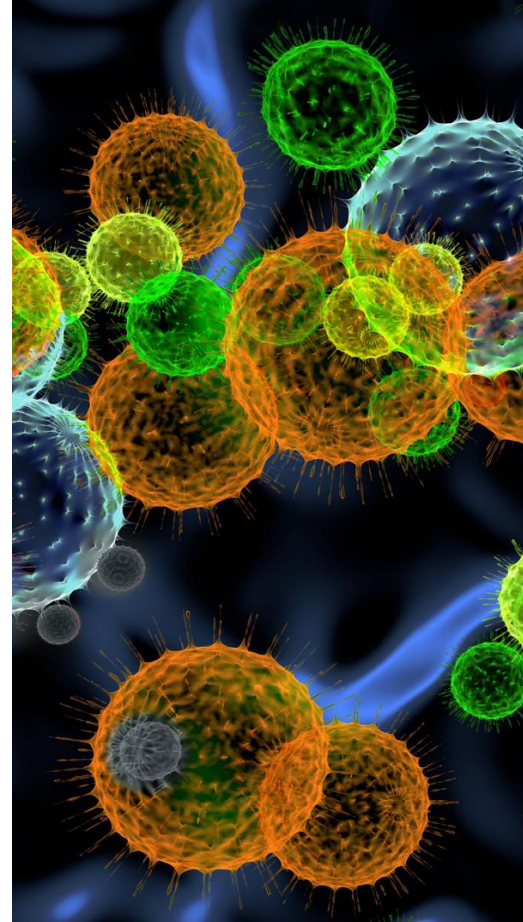
M-protein, monoclonal protein; MR, minimal response; PD, progressive disease; PR, partial response; sCR, stringent complete response; SD, stable disease; VGPR, very good partial response.

Source: Mateos MV, et al. ASH 2019. #1883

# Encouraging data for melflufen in combination with daratumumab

## Summary of combination with daratumumab – n=33

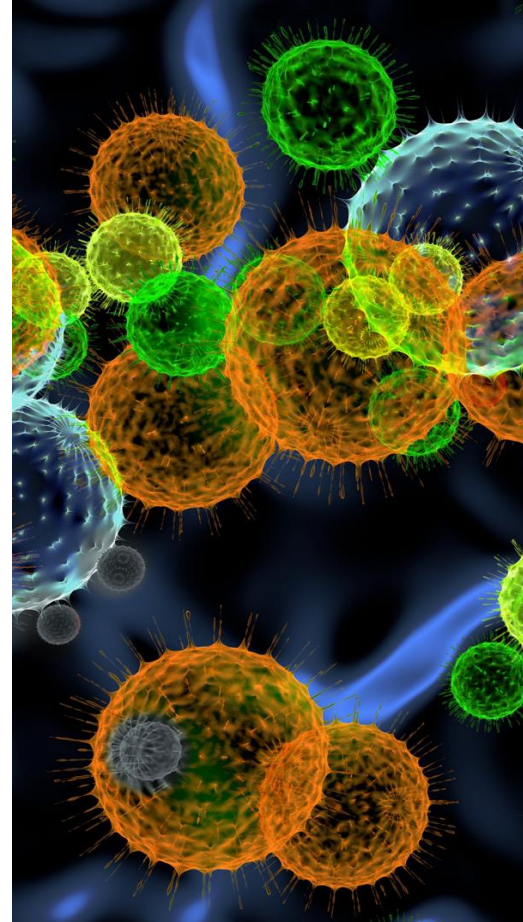
- Median of 2 prior lines of therapy
- True RRMM population (not maintenance refractory) – 39% had disease progression while on last line of therapy and 60% high-risk cytogenetics
- **ORR of 76%** with good tolerability and deepening responses - 22 patients ongoing
- Median **PFS of 14.3 months**



# Emerging data for melflufen in combination with bortezomib

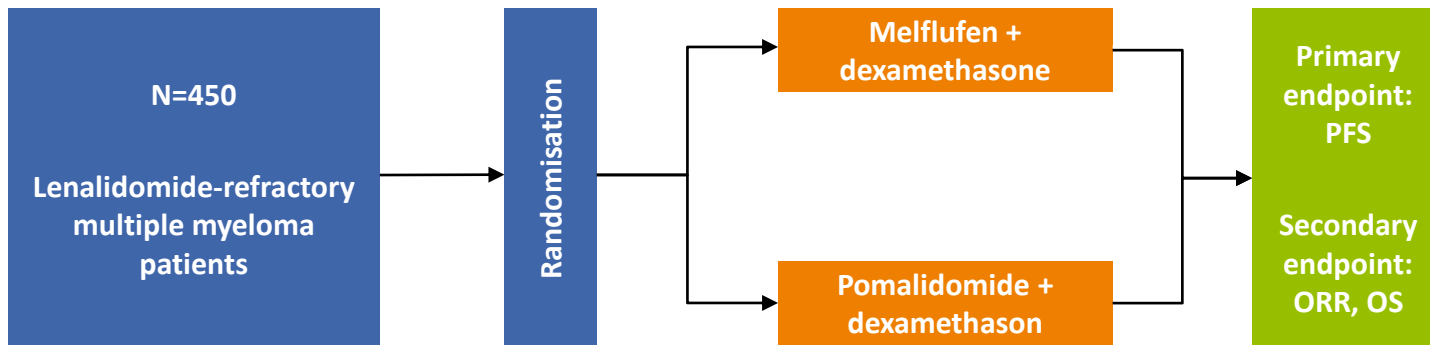
## Summary of combination with bortezomib – n=6

- Elderly population – Median of 2.5 prior lines of therapy
- True RRMM population (not maintenance refractory) – 50% had disease progression while on last line of therapy
- 4/6 responded on therapy (**ORR 67%**) with good tolerability and deepening responses – 3 pts ongoing
- Median PFS not reached with the longest patient on treatment for 16 months





# Data to date provide high conviction for success in our ongoing phase 3 trial OCEAN



## 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

# The phase 3 combination trial LIGHTHOUSE will be of high strategic importance

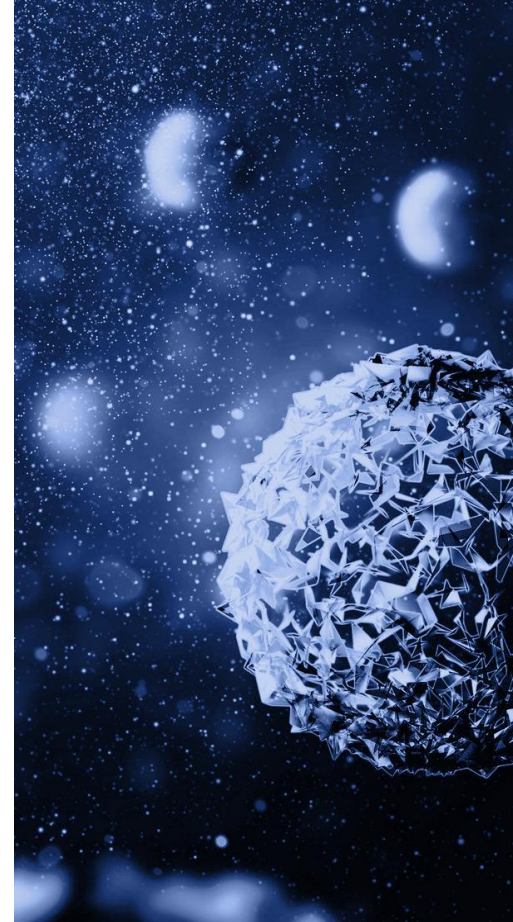
## Second phase 3 trial with melflufen in multiple myeloma

- Melflufen + daratumumab vs daratumumab randomized 2:1
- Subcutaneous administration of daratumumab

## Two objectives:

- Expand market potential – extend label with melflufen in combination with daratumumab in earlier line patients
- De-risk the development program – add a third trial that can result in market registration in the EU and US

**We are in final preparations of the study and aim to start the study early 2020**



# Our new indication AL Amyloidosis

**Similarly to myeloma**, AL amyloidosis is a disease of the B-cell system

- Antibody light-chains misfold and form deposits in multiple organs with organ dysfunction as a result
- Orphan disease - 30-45,000 patients in the USA and the EU<sup>1</sup>
- Majority of patients >65 years old

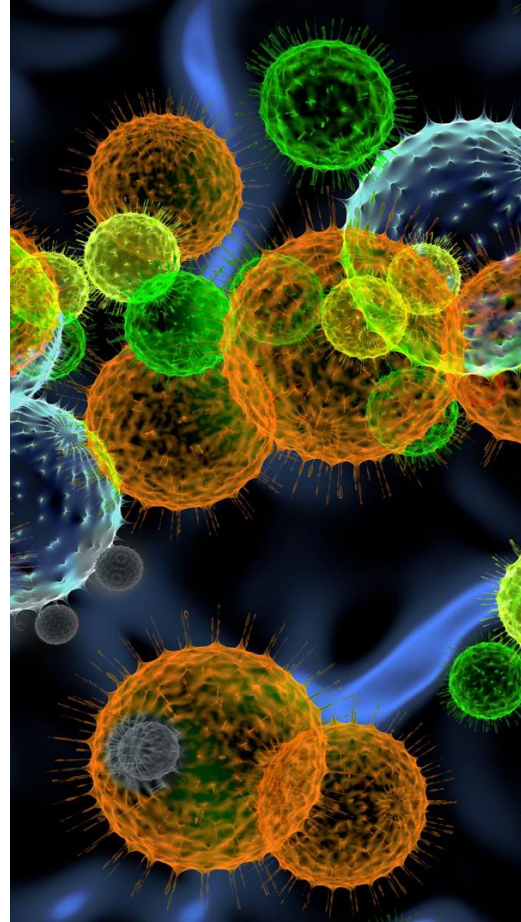
**Similar drug use as for myeloma** – drugs that are efficacious in myeloma are most of the time also used in AL amyloidosis

**Limited treatment options** - with median overall survival of 1.5-2.0 years (1995-2013) with a trend towards improved survival (3.5 years for the period 2010-2013)<sup>2</sup>

**Strong pre-clinical data presented at ASH 2019 with distinct differentiation vis-à-vis other drugs including alkylating agents** – pre-clinical data translates well in AL amyloidosis

**Phase I+II study** – up to 45 patients across both phases

**The study to start imminently, patient screening ongoing**



# The coming quarters will be very information rich

	Dec 2019	Q1 2020	Q2 2020	Q3 2020	Q4 2020
✓	Data from HORIZON, ANCHOR at ASH	FPI LIGHTHOUSE	LPI BRIDGE	Top-line results OCEAN	Potential accelerated approval in US
		LPI OCEAN	New data and updates at EHA	LPI ANCHOR	Potential Launch in US
		FPI Amyloidosis Trial	NDA submission		

# Summary

## Significant unmet needs in Multiple Myeloma

- \$17 B orphan market

## Melflufen has the potential to become a new treatment backbone for relapsed refractory multiple myeloma

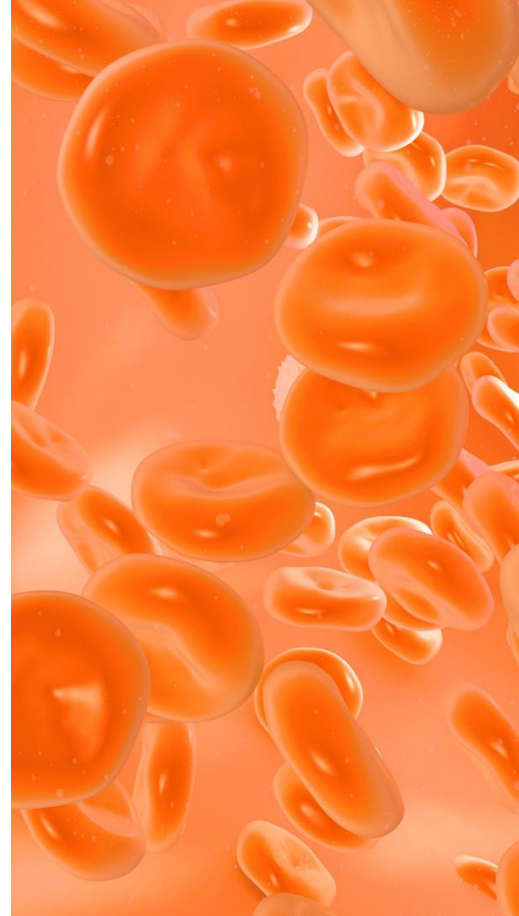
- Phase 2 study, O-12-M1, showed very strong survival data
- Both phase 2 studies, HORIZON and ANCHOR show strong overall response (ORR) data and competitive profile for progression free survival (PFS)
- Generally well tolerated giving patients good quality of life

## Late stage development program with multiple ways to get approval

- Submission for accelerated approval for triple-class refractory patients in the US targeted during H1 2020 based on HORIZON data
- Phase 3-trial OCEAN expected to be fully enrolled Q1 2020
- Additional Phase 3-trial, LIGHTHOUSE will start early 2020

## Strong financial position

- Cash position SEK ~1.1 B (\$ 118 M) as of September 30



***Thank you for  
your attention!***

