

A photograph of a male doctor with a grey beard and glasses, wearing a white lab coat over blue scrubs, with a red stethoscope around his neck. He is looking down at an elderly male patient lying in a hospital bed. The patient is wearing a white hospital gown. The background is a bright, modern hospital room with large windows and greenery visible outside.

**Oncopeptides**

**Company presentation October, 2019**



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# Oncopeptides at a glance

## Develops targeted cancer treatments

- Proprietary peptidase-enhanced 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 Q1-20 based on ongoing phase 2 study HORIZON
- Triple-class refractory MM

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

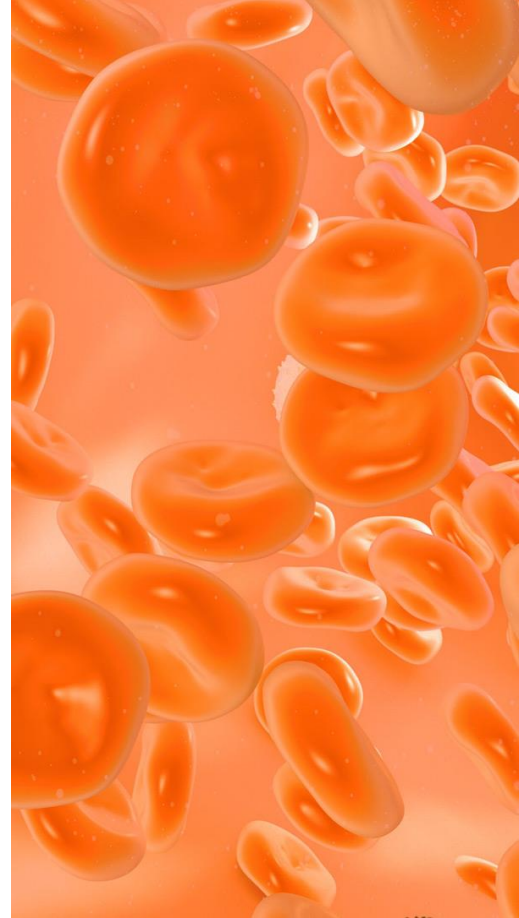
- Approximately 450 patients at 140 sites
- Two additional supporting trials ongoing, additional Phase 3 to be started around year-end

## Listed on NASDAQ Stockholm, strong financial position

- Market cap: SEK 6.2 B (\$ 625 M)
- Cash position: SEK 627 M (\$ 64 M) as of June 30, SEK 683 M (\$ 69 M) raised in early July

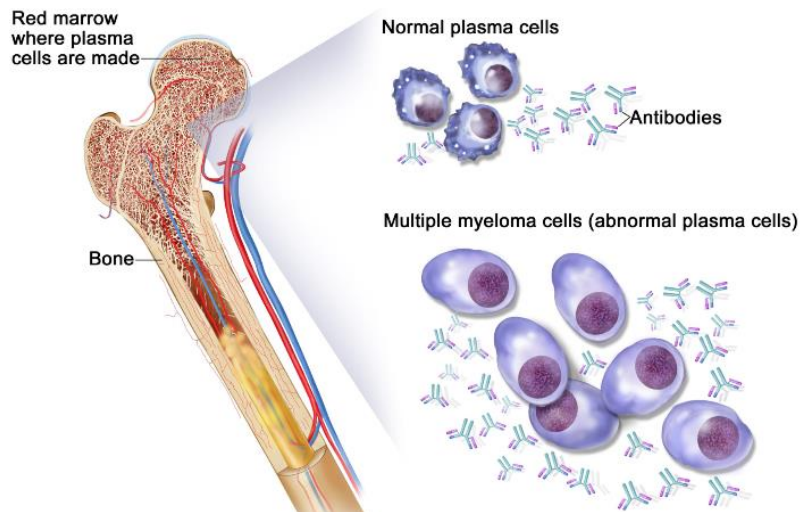
## New indications and NCEs in development

- Clinical trials expected to start in 2019

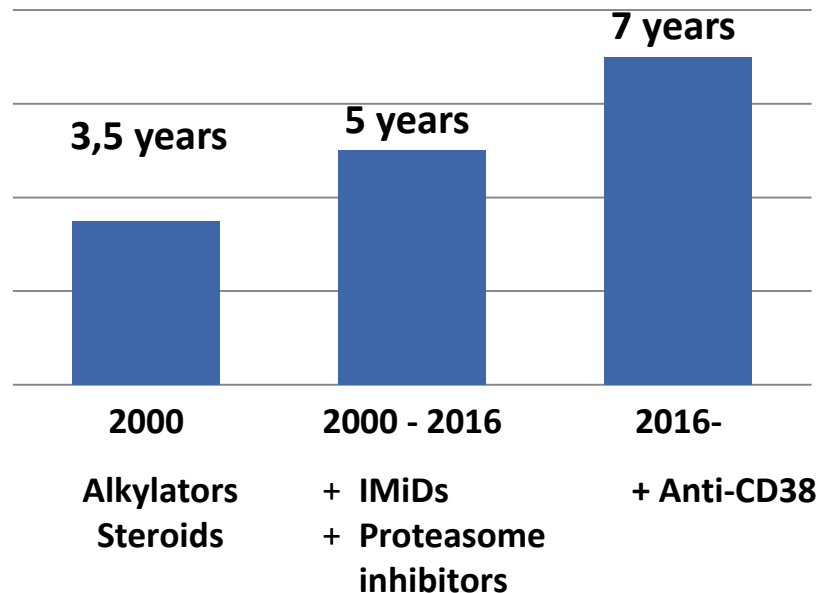


# Multiple Myeloma is a hematological cancer without cure

## Myeloma – Uncontrolled plasma cell proliferation

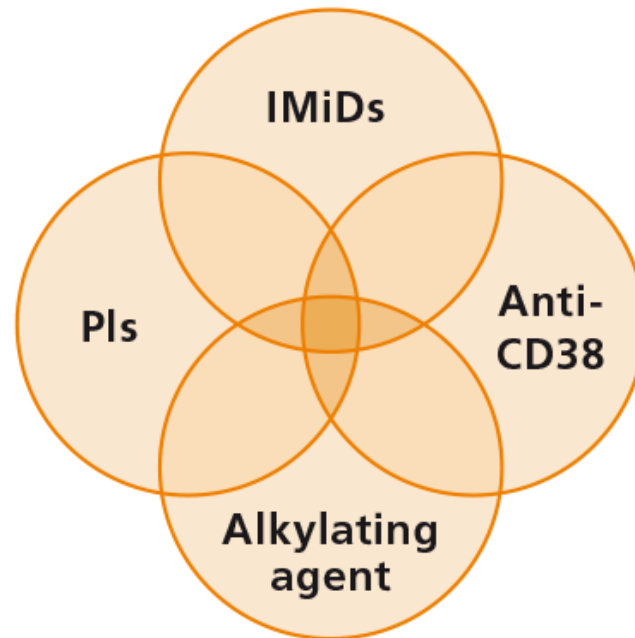


## Median Survival increasing with more available treatment options



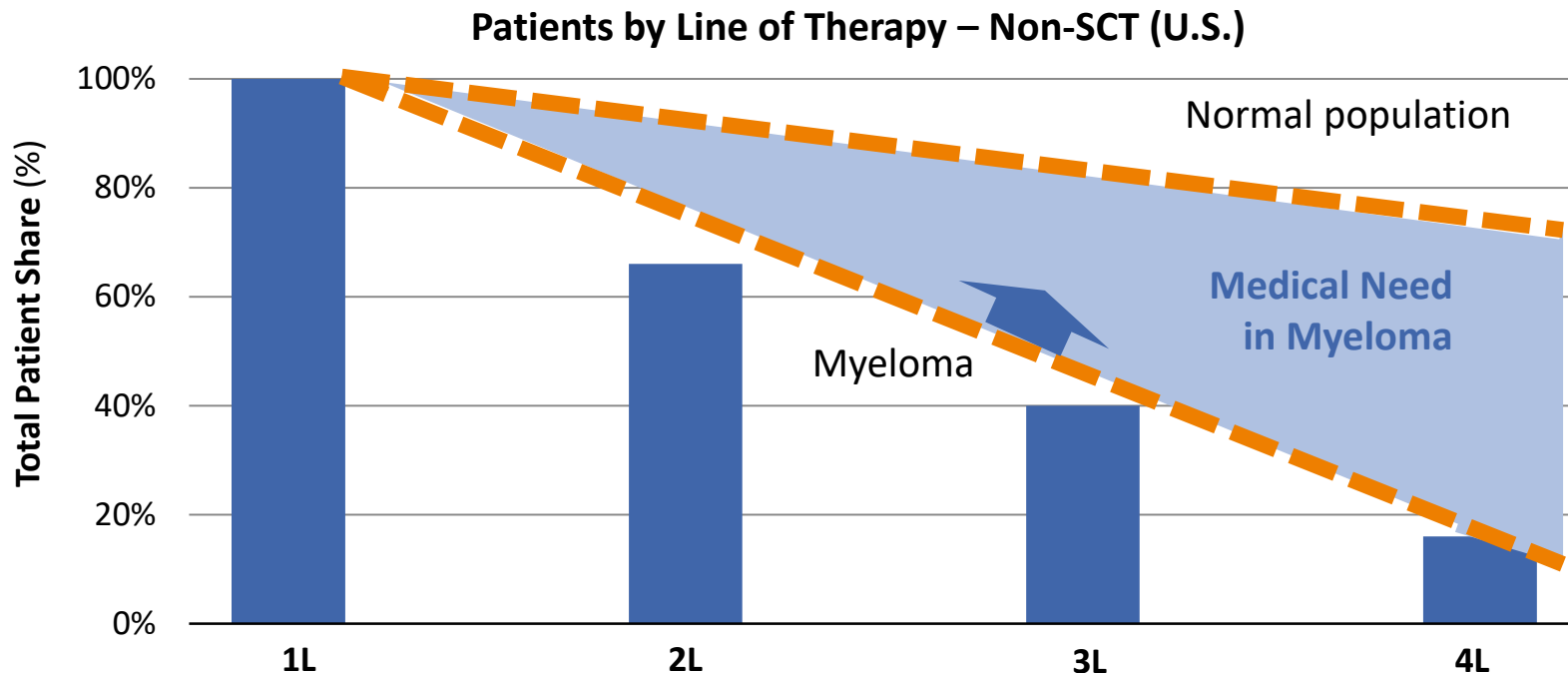
# Significant medical needs remain

- 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



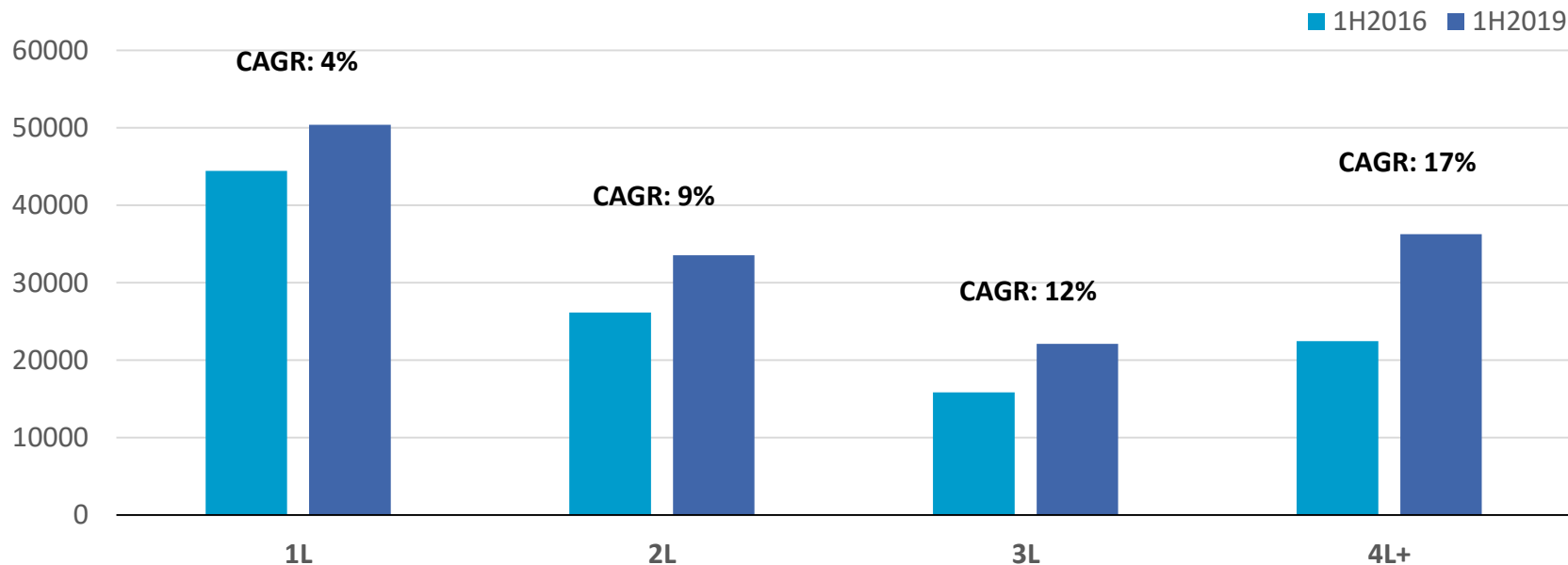
# We are still far from making myeloma a chronic disease

- 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

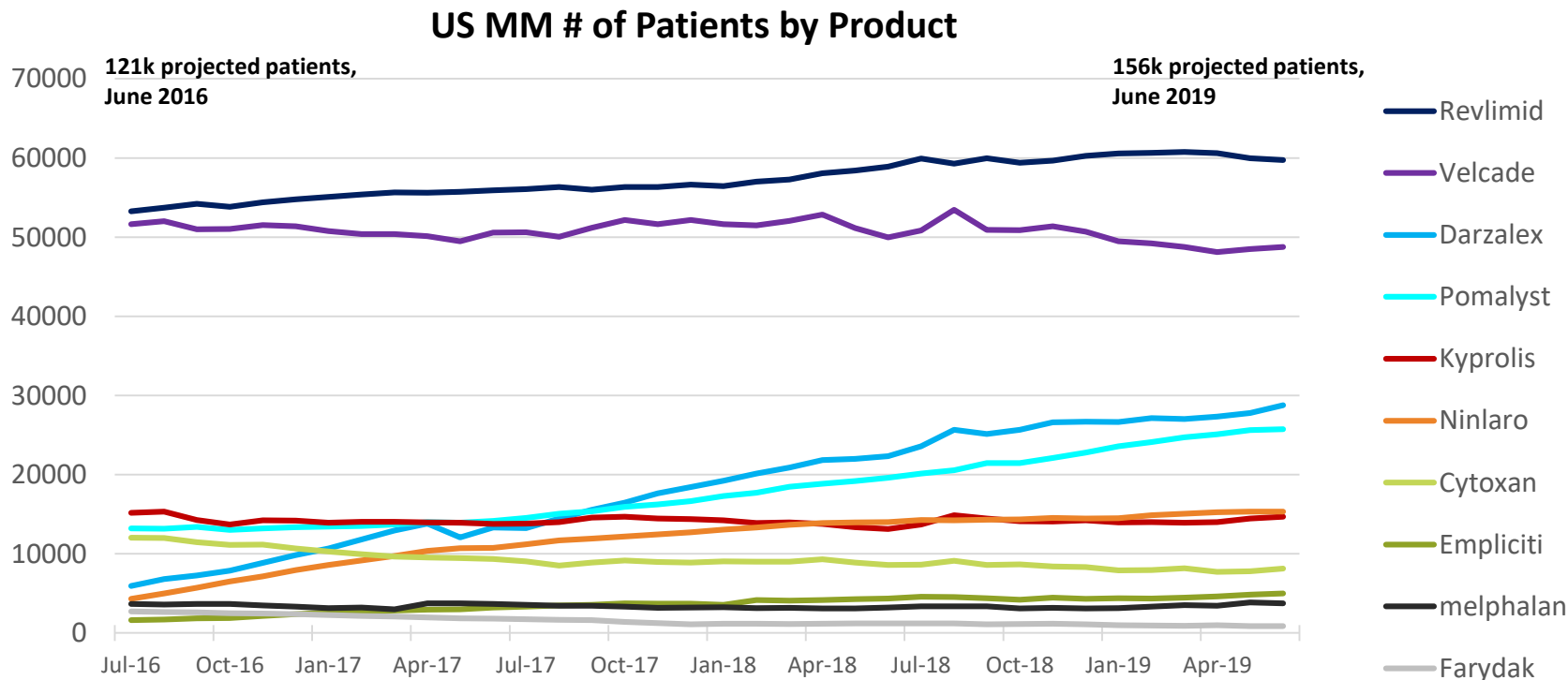
Projected US multiple myeloma patients by line of therapy



Source: Intrinsiq MAT, June 2019

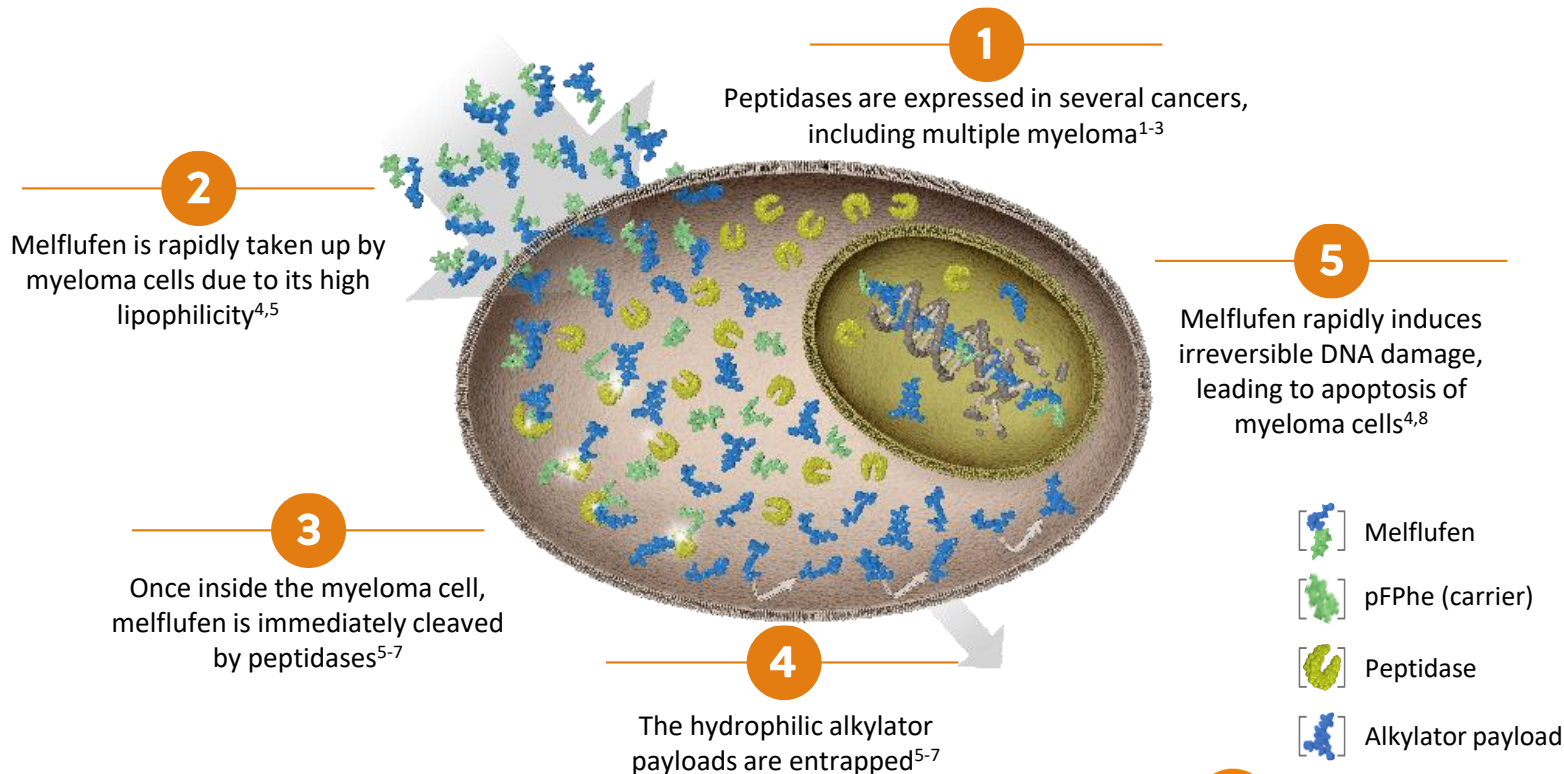
Note: 3-yr annual growth rate for 1H2016-2H2019

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



# Melflufen is a first in class peptide-conjugated alkylator

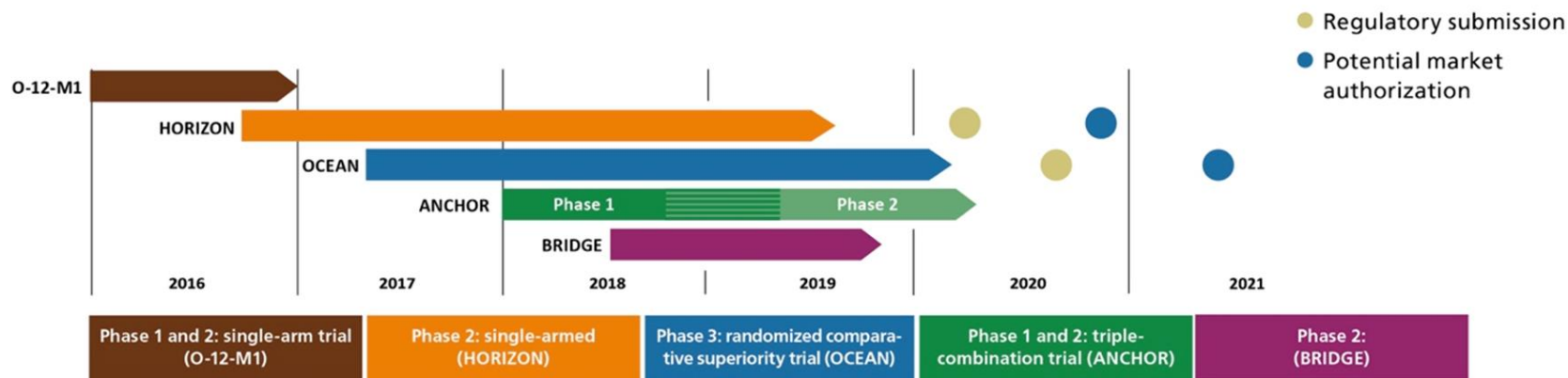
- Uses high peptidase levels to target myeloma cells



Sources: 1. Hitzert SM, et al. *Amino Acids*. 2014;46:793-808. 2. Moore HE, et al. *Mol Cancer Ther*. 2009;8:762-770. 3. Wickström M, et al. *Cancer Sci*. 2011;102:501-508. 4. Chauhan D, et al. *Clin Cancer Res*. 2013;19:3019-3031. 5. Wickström M, et al. *Oncotarget*. 2017;8:66641-66655. 6. Wickström M, et al. *Biochem Pharmacol*. 2010;79:1281-1290. 7. Gullbo J, et al. *J Drug Target*. 2003;11:355-363. 8. Ray A, et al. *Br J Haematol*. 2016;174:397-409.



# 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**

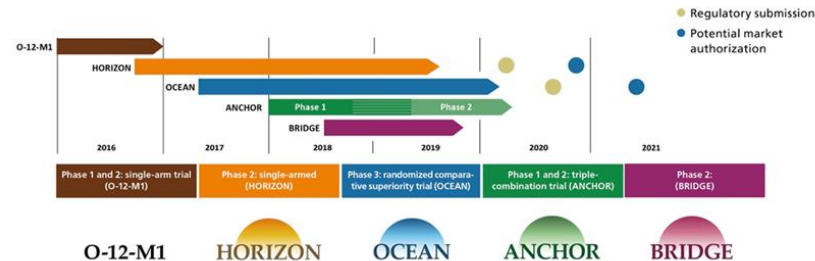
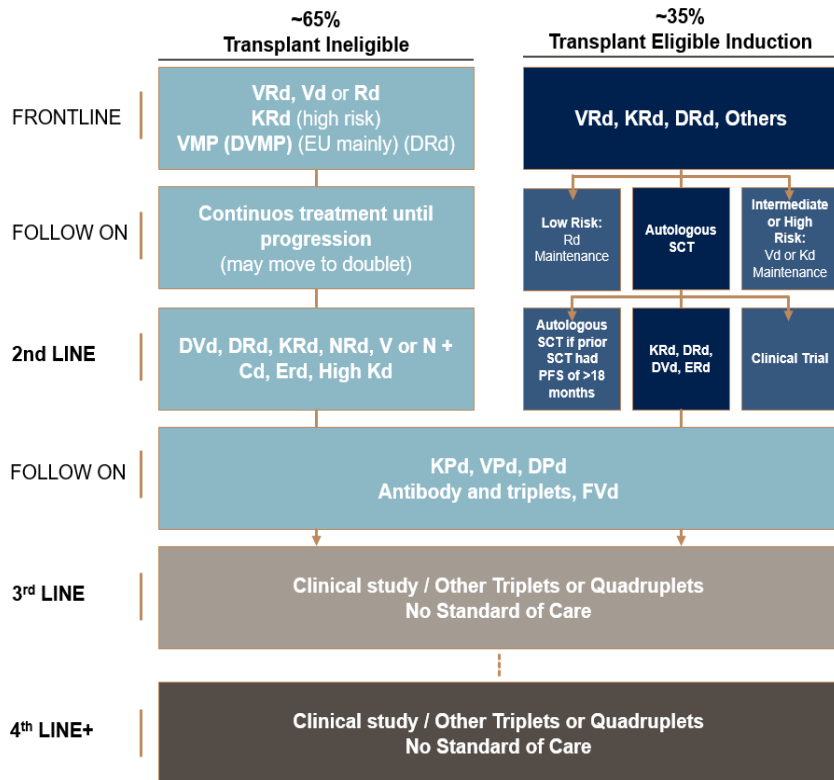
Show combination synergy and tolerability with daratumumab and bortezomib

**BRIDGE**

Show that melflufen can be used in patients with renal impairment



# Clinical program covers entire relapsed setting



# 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



Comorbidity or tolerability limitations



Limited to no single agent data



# Summary of key late stage development programs in RRMM – all new mechanisms have safety issues

Name	Company	MoA	Phase	Patient population	Efficacy*	Safety	Estimated approval
Selinexor	Karyopharm	SINE, XPO1	Approved Jul'19	Triple refractory	ORR: 26% PFS: 3.7mo	GI toxicity, cytopenia, dose modifications	N.A.
Daratumumab SC	J&J/ Genmab	aCD38 Mab	III	3+ prior lines (may expand to all Dara IV indications)	ORR: 41% SC vs. 37% IV	No new safety signals vs. IV	1H20
Isatuximab	Sanofi	aCD38 Mab	III	2+ prior lines	ORR: 24% PFS: 18.7mo	Infusion site reactions, cytopenia	1H20
Venetoclax	Abbvie/ Roche	BCL-2	III	1-3 prior lines	ORR: 21%	Deaths, cytopenia	Clinical hold lifted Jun'19 for t(11;14)
bb2121	Bluebird/ Celgene	BCMA CAR-T	II	3+ prior lines	ORR: 85% PFS: 11.8mo	Cytokine release syndrome, cytopenia	2H20
GSK916	GSK	BCMA ADC	II	3+ prior lines	ORR: 60% PFS: 12mo	Blurred vision, cytopenia	2H20

\* Latest data cut for single agent + dexamethasone trials

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

## 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

## MELFLUFEN

O-12-M1 showed an ORR of 31% and HORIZON an ORR of 27% 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)

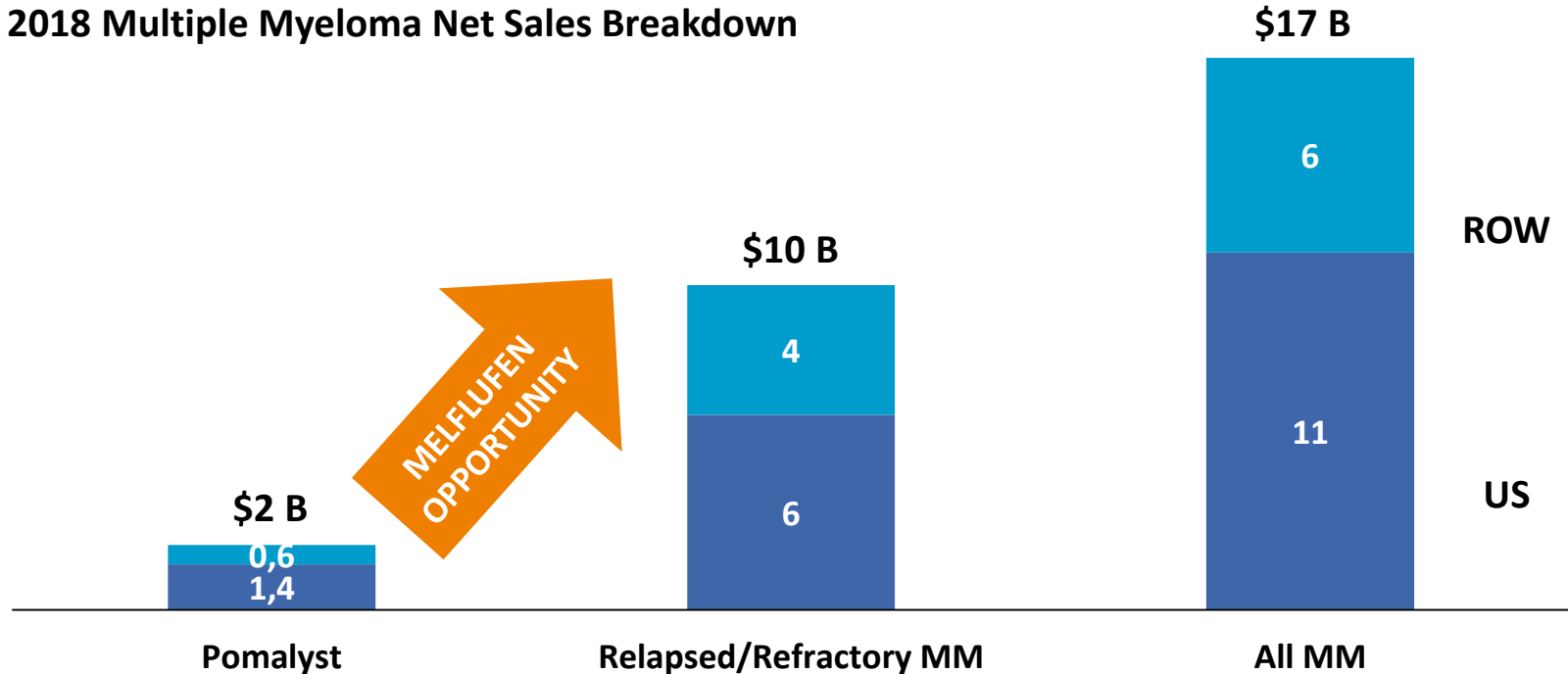
Good QoL with almost no non-hematological AEs

No co-morbidity or drug-drug interactions limitations

One 30-minute infusion every 28 days

# Melflufen opportunity in Relapsed Refractory Multiple Myeloma

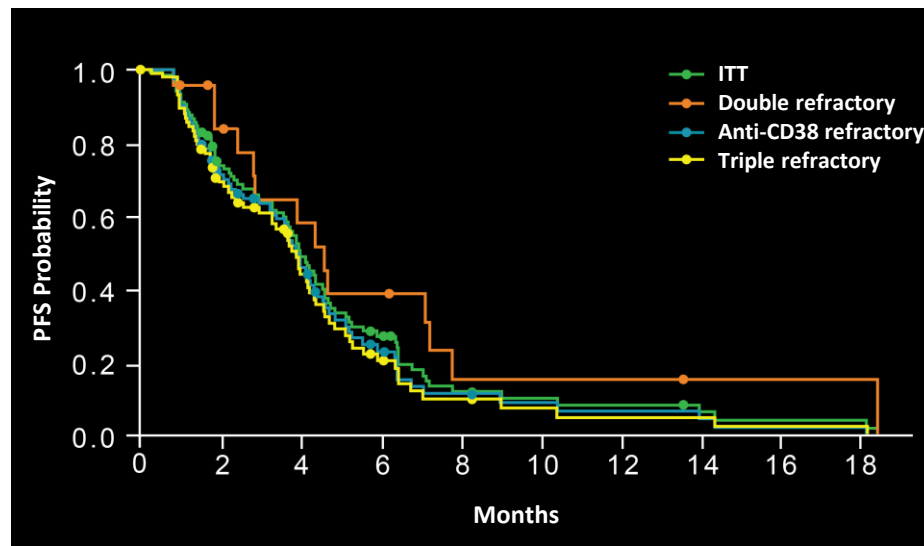
– 2018 Multiple Myeloma Net Sales Breakdown



# Promising efficacy data for patients without remaining treatment options presented at EHA

Response	NE	PD	SD	MR	ORR	sCR	VGPR	PR
%	1%	13%	46%	12%	28%	1%	9%	19%

- n=121, 5-6 prior lines of therapy (median of 5)
  - 62% of patients had high-risk cytogenetics
  - 44% had extramedullary disease (EMD) at screening
  - 74% were triple-class refractory
- Strong overall response rate of 28%
- Median Progression Free Survival of 4.0 months
- Strong activity in triple-class (IMiD, PI and daratumumab) refractory patients
  - 20% ORR at latest cut



# Strong activity in relapsed patients with extramedullary disease presented at IMW

## Extramedullary disease occurs when myeloma cells form tumors outside the bone marrow

- Outcomes remain very poor for patients with EMD
- Incidence approximately 10-15% reported at relapse, increasing with reported rates up to 40%

## Other studies have failed to demonstrate substantial response in relapsed EMD

- Only daratumumab and pomalidomide have shown any responses
- ORRs of 17% and 9%, respectively in less ill patients

## EMD data from HORIZON presented at IMW, Sep 15

- 44 EMD patients, largest EMD cohort ever
- Late stage patients, median of 5 prior lines and 5.5 years since diagnosis

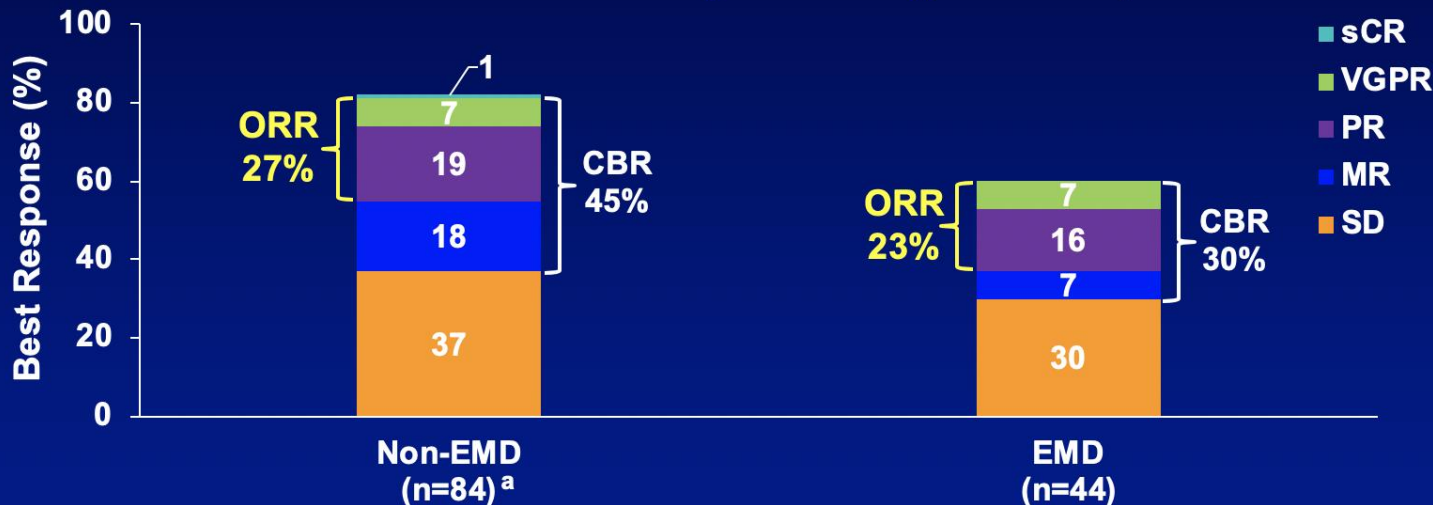
## High response rate and highly relevant responses

- 23% ORR for EMD patients, similar to non-EMD
- Survival benefit >12 months for EMD responders vs non-responders

HORIZON data presented at IMW Sep, 2019  
(n=128)

	EMD-relapsed patients (n=44)	Non-EMD relapsed patients (n=84)
Overall response rates, %	23	27
Duration of response, months	3.4	4.4
Median overall survival responders, months	18.5	17.2
Median overall survival non-responders, months	5.1	8.5

# Overall Response (n=128)

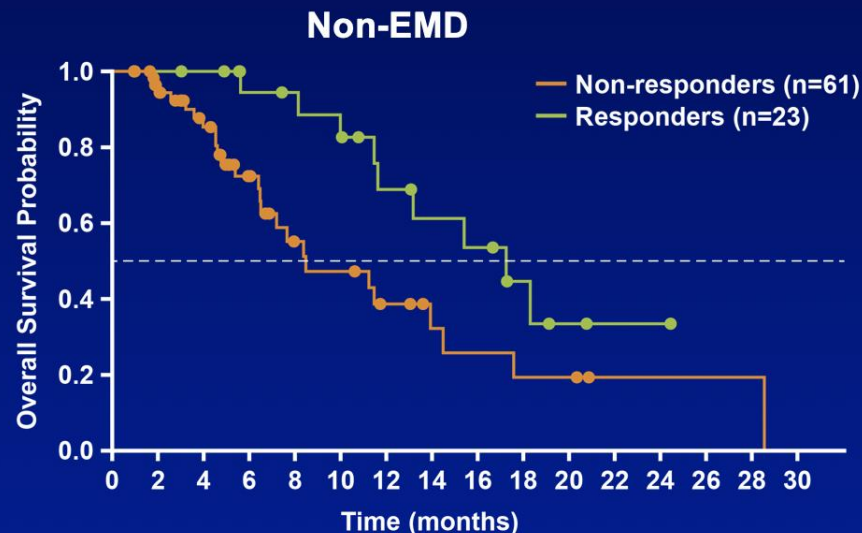
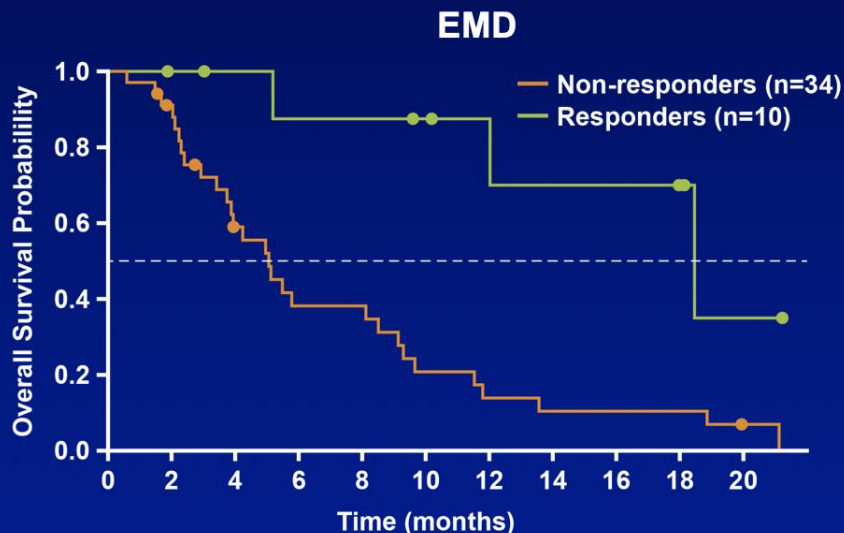


- Similar ORR in non-EMD and EMD pts, with an ORR of 27% and 23% respectively
  - Investigator-assessed response<sup>1</sup>
  - IRC review ongoing
- Median DOR for non-EMD pts 4.4 mos (95% CI, 3.5-11.2)
- Median DOR for EMD pts 3.4 mos (95% CI, 1.8-15.4)

<sup>a</sup> Two non-EMD pts with pending response information available at data cut off 30<sup>th</sup> July 2019.

1. Rajkumar SV, et al. *Blood*. 2011;117:4691-4695.

# OS in EMD and Non-EMD Pts Stratified by Response



- Median OS in EMD responders vs. non-responders: 18.5 vs. 5.1 mos
- Median OS in Non-EMD responders vs. non-responders: 17.2 vs. 8.5 mos
  - Similar trend for PFS in responders vs. non-responders: 4.8 vs. 2.2 mos in EMD pts; 6.4 vs. 3.8 mos in non-EMD pts
- 54% of ITT pts received subsequent therapy with no significant difference in outcome between EMD vs. non-EMD pts<sup>1</sup>

Data cutoff 30 July 2019.

1. Gandhi UH, et al. *Blood*. 2018;132(suppl 1):Abstract 3233.

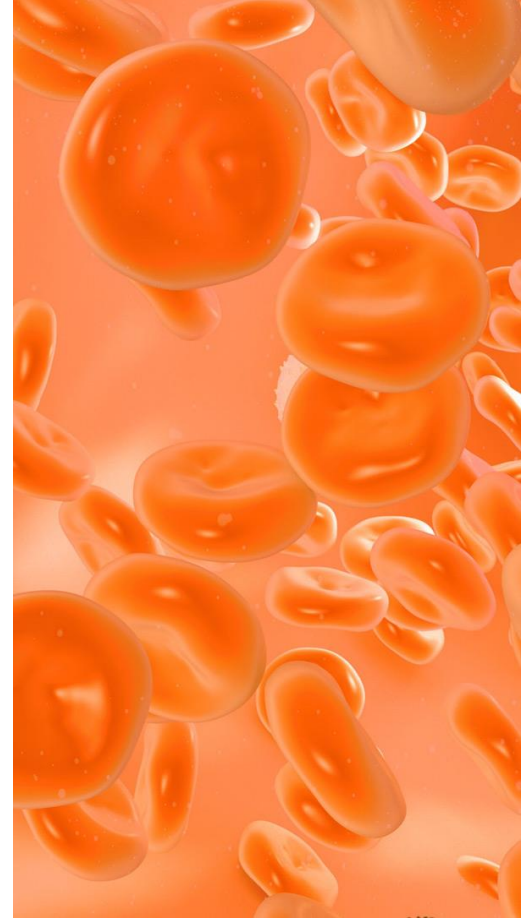
# Safety indicates a very good quality of life profile for patients

- Absence of grade 3 and 4 TEAEs outside of the hematological system and infections and infestations
- Low infection rate in comparison with other myeloma drugs
- Hematological toxicity clinically manageable
  - 78% of patients in HORIZON maintain the full 40 mg dose despite low bone marrow reserves

Grade 3 and 4 TEAEs occurring in >5% of patients	
	HORIZON
SAE rate	40%
Hematological	
Anemia	30%
Neutropenia	57%
Thrombocytopenia	58%
Febrile neutropenia	7%

# Application process initiated for accelerated approval in the US based on HORIZON

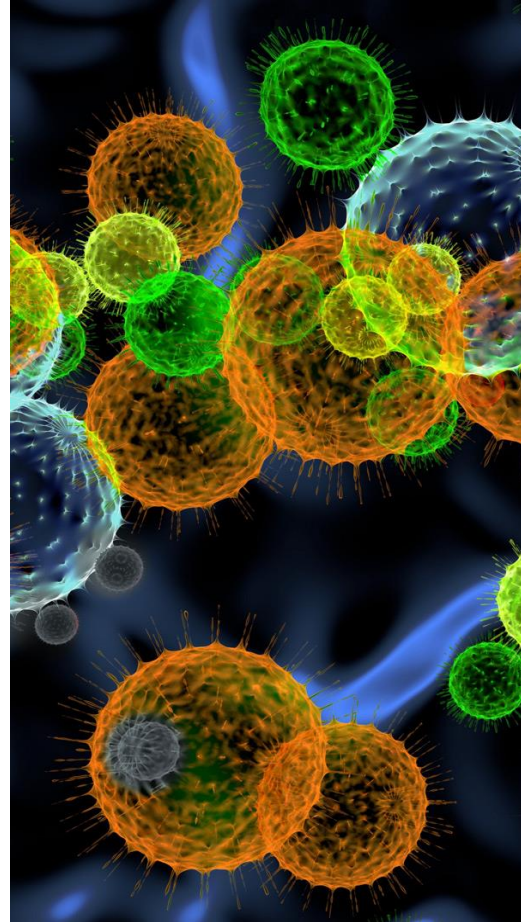
- Oncopeptides was engaged in dialogue with the FDA during the spring of 2019 about the HORIZON data
- FDA had access to all data from our ongoing and completed trials (apart from OCEAN)
- Based on the dialogue, Oncopeptides has initiated the submission process for accelerated approval in the US
  - Treatment of relapsed refractory multiple myeloma patients whose disease is triple-class refractory (i.e. refractory to one IMiD, one PI and one anti-CD38 Mab)
- Target filing date is Q1 2020 with possible US launch late 2020



# Data indicates synergistic effect of Melflufen+Daratumumab combination

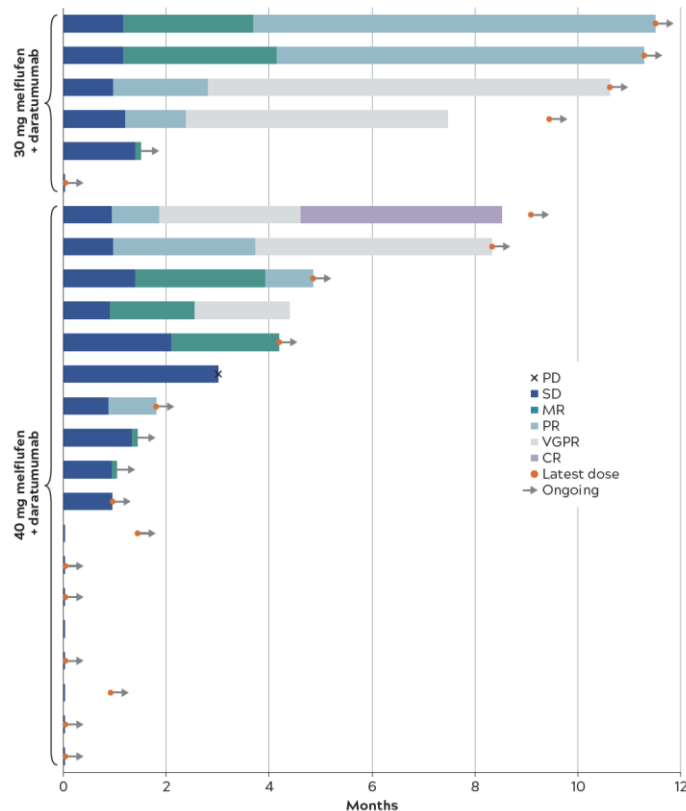
## Summary of combination with daratumumab – n=24

- 2-3 prior lines of therapy
- True RRMM population (not maintenance refractory) – 50% had disease progression while on last line of therapy and 37% high-risk cytogenetics
- **ORR of 82%** with good tolerability and deepening responses
- Median PFS not reached with longest patient on treatment for 12 months. All patients apart from one ongoing.



# Encouraging data for Melflufen+Daratumumab combination presented at EHA

## Deepening responses – all but one patient ongoing



## Patient characteristics

Characteristics	30 mg* (n=6)	40 mg (n=18)
Median age, years (range)	57.0 (49-78)	62.0 (35-77)
Gender, n (%)		
Male/female	3 (50)/3 (50)	13 (72)/5 (27)
Median time since diagnosis, years (range)	3.1 (1.9-8.0)	4.4 (0.7-8.2)
Median number of previous lines (range)	2.5 (1-3)	2 (1-4)
Prior ASCT/alkylator exposed, n (%)	5 (83)/3 (50)	14 (78)/10 (56)
Alkylator refractory, n (%)	1 (17)	4 (22)
IMiD refractory, n (%)	3 (50)	11 (61)
PI refractory, n (%)	0	10 (56)
Last-line refractory, n (%)	2 (33)	10 (56)
IMiD + PI refractory, n (%)	0	8 (44)
ISS at study entry, <sup>b</sup> n (%)		
I/II/III	6 (100)/0/0	13 (76)/2 (12)/2 (12)
High-risk cytogenetic by FISH, <sup>c</sup> n (%)	2 (40)	5 (36)
Median albumin level, g/dL (range)	4.1 (3.1-4.5)	3.9 (3.1-4.9)

## Treatment-related Grade 3/4 AEs

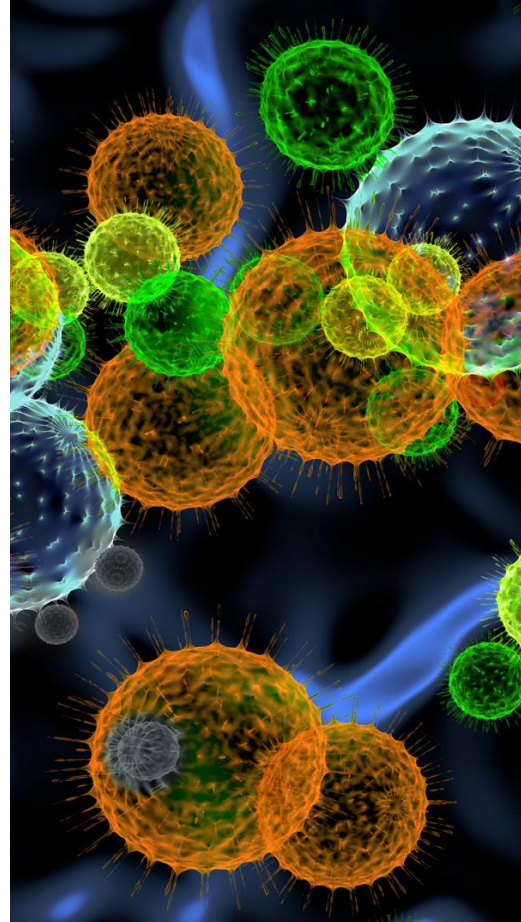
Preferred term	No. of Patients (%)	
	30 mg (n=6)	40 mg (n=18)
Any AE	5 (83)	14 (78)
Neutropenia <sup>a</sup>	5 (83)	10 (56)
Thrombocytopenia <sup>a</sup>	3 (50)	11 (61)
Anemia	2 (33)	1 (6)
Febrile neutropenia	1 (17)	0
Fatigue	0	1 (6)
Agitation	0	1 (6)
Muscular weakness	0	1 (6)



# Data indicates synergistic effect of Melflufen+Bortezomib combination

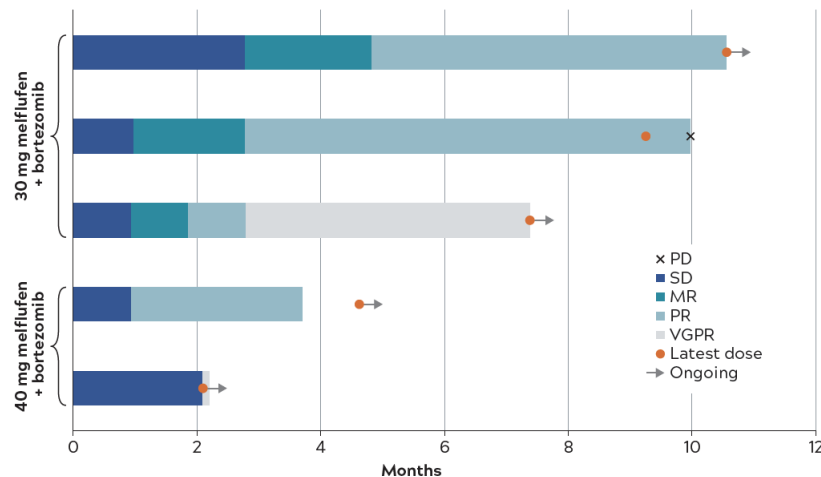
## Summary of combination with bortezomib – n=5

- Elderly population – 2-3 prior lines of therapy
- True RRMM population (not maintenance refractory) – 50% had disease progression while on last line of therapy
- 5/5 responded on therapy (**ORR 100%**) – all pts ongoing apart from one with good tolerability
- Median PFS not reached with the longest patient on treatment for 11 months



# Encouraging data for Melflufen+Bortezomib combination presented at EHA

## Deepening responses – all but one patient ongoing



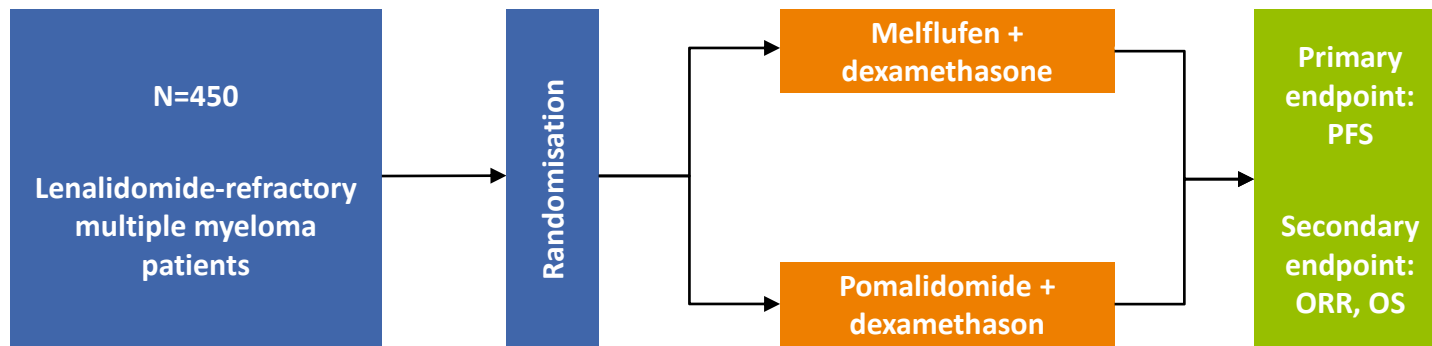
## Patient characteristics

Characteristics	n=5 <sup>a</sup>
Median age, years (range)	73.0 (63-82)
Gender, n (%)	
Male/female	3 (60)/2 (40)
Median time since diagnosis, years (range)	5.8 (1.2-7.4)
Median number of previous lines (range)	2 (2-4)
Prior ASCT/alkylator exposed, n (%)	1 (20)/4 (80)
Alkylator refractory, n (%)	1 (25)
PI exposed, n (%)	5 (100)

## Treatment-related Grade 3/4 AEs

Preferred Term	No. of Patients (%)	
	30 mg (n=3)	40 mg (n=2)
Any AE	2 (67)	1 (50)
Thrombocytopenia <sup>a</sup>	2 (67)	1 (50)
Neutropenia <sup>a</sup>	2 (67)	0
Pneumonia <sup>a</sup>	1 (33)	0

# 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

# Pomalidomide shares resistance mechanism with lenalidomide

## Average IMiD free period was significant in pomalidomide registration study

- Only 29% received lenalidomide as last treatment

## Lenalidomide used more aggressively today

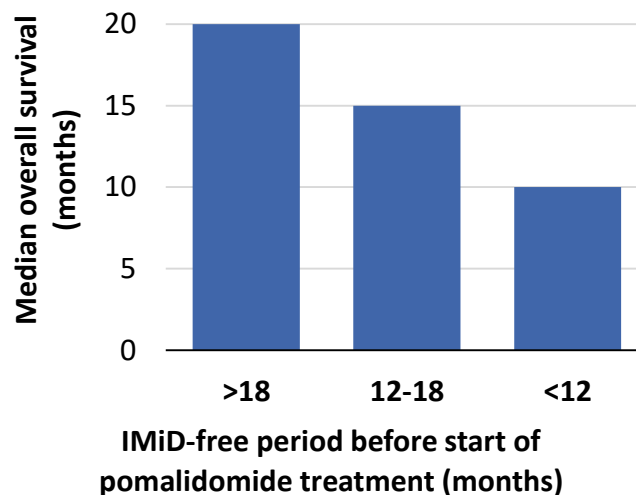
- Median maintenance duration 24 months instead of 10 months

## In OCEAN all patients have failed on lenalidomide within 18 months

- Vast majority has lenalidomide as last treatment

## No assumptions have been made in OCEAN power calculation to account for increased cross resistance

## Pomalidomide efficacy decreases for recent lenalidomide failures



# Our new pivotal combination trial LIGHTHOUSE of high strategic importance

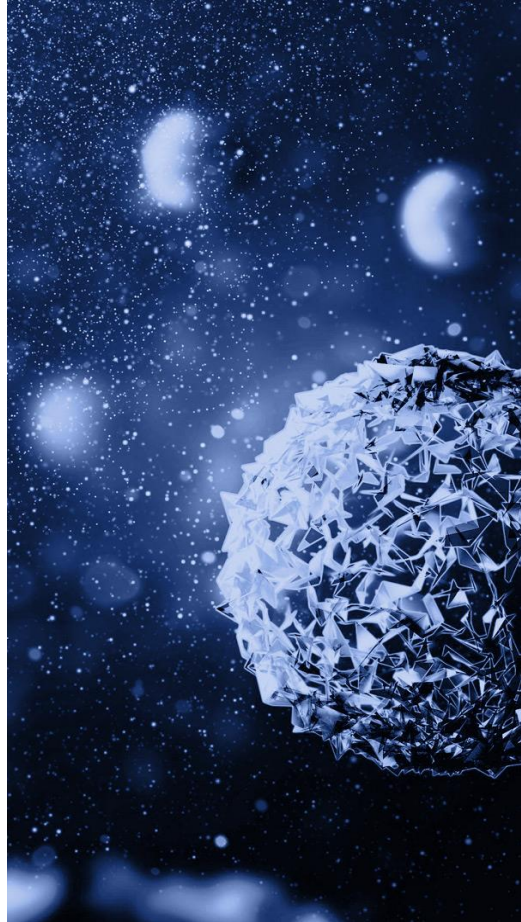
## Second pivotal phase III trial with melflufen in multiple myeloma

- Melflufen+daratumumab+dexamethasone vs daratumumab+dexamethasone randomized 2:1

### 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 preparing the study and aiming for enrolling the first patient around year-end 2019**



# Our new indication AL Amyloidosis

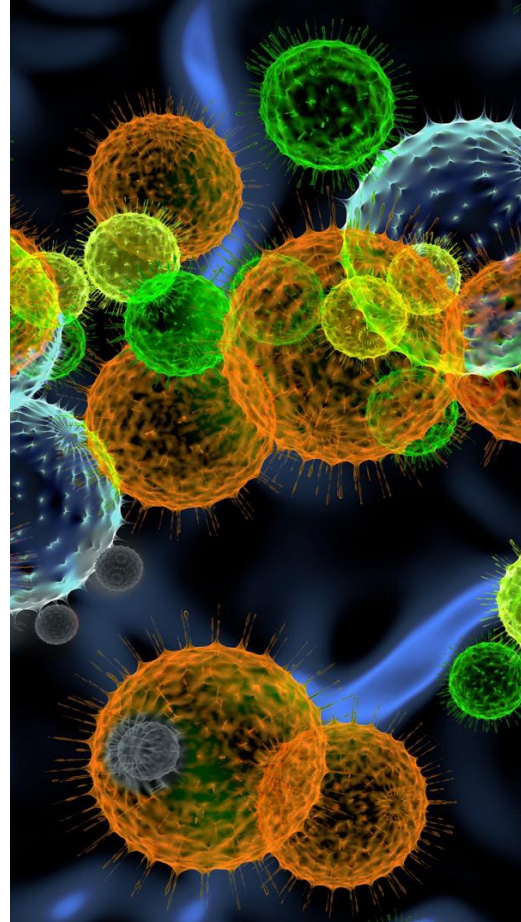
**Similar to myeloma**, AL amyloidosis is a disease of the B-cell system

- 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

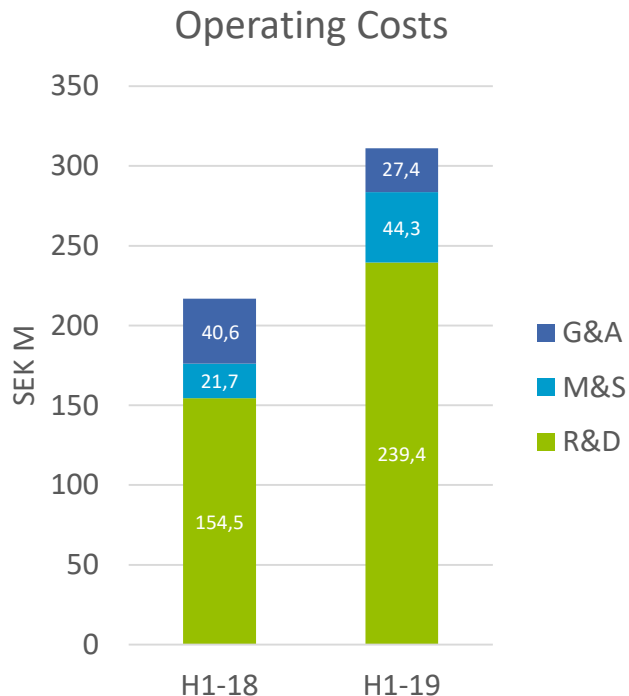
**Similar drug use as for myeloma** – drugs that are efficacious in myeloma are also most of the time efficacious in AL amyloidosis

**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>

**Phase I+II study** with first-patient-in H2 2019 – up to 30 patients across both phases



# Financial results for the period Jan – Jun 2019



- Operating loss increased to SEK 305.6 M (loss:205.1)
  - R&D increase primarily due to increase in Clinical & drug supply: SEK 201.4 M (135.5)
    - OCEAN costs SEK 110.7 M (69.4)
    - HORIZON costs SEK 29.5 (12.6)
    - ANCHOR costs SEK 19.4 M (12.0)
  - Build-up of commercial and medical relations explains increase in M&S costs
- Operating costs include non-cash costs related to incentive programs
  - SEK 17.8 M (50.2) for H1
- Cash flow from operating activities neg. SEK 265.8 M (neg. 130.6)
- Cash position was SEK 626.8 M (568.2) as of June 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

# The next 12 months represents the most information rich period in Oncopeptides' history

H2 2019	H1 2020
FPI Amyloidosis Trial	NDA submission
FPI LIGHTHOUSE	LPI OCEAN
✓ LPI HORIZON	LPI ANCHOR
LPI BRIDGE	Top-line results OCEAN
✓ Updated Data from HORIZON on EMD patients at IMW	
Updated Data from HORIZON, ANCHOR and BRIDGE at ASH	

# 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

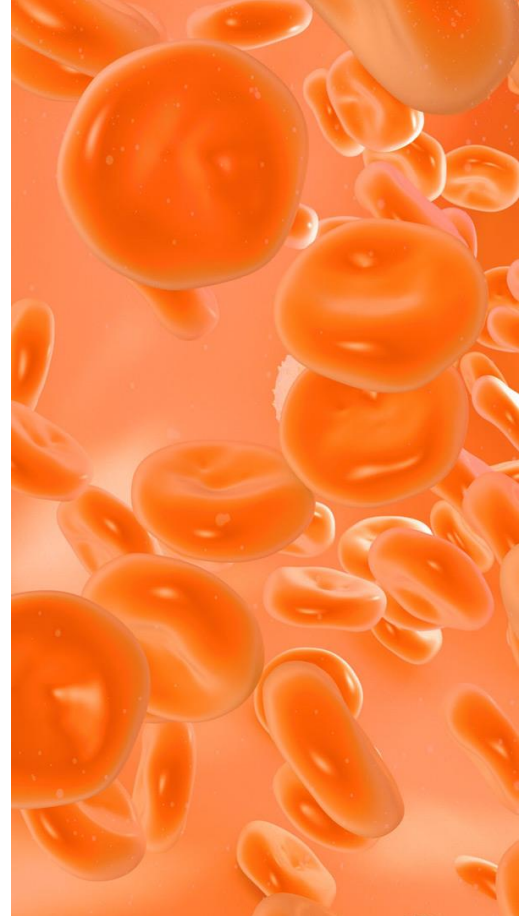
- Phase 2 study, O-12-M1, showed very strong survival data
- Generally well tolerated giving patients good quality of life

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

- Submission for accelerated approval for triple-class refractory patients in the US targeted in Q1-20
- Pivotal phase 3 expected to be fully enrolled Q1 2020
- Additional Phase 3 to be started around year-end 2019

## Strong financial position

- Cash position SEK ~1.3 B (\$ 130 M) after share issue early July



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

