AXL inhibitors as cornerstone of combination cancer therapy

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January 8–10, 2018
Richard Godfrey, CEO

BerGenBio

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Corporate snapshot

Background

Leaders in developing therapeutics that target AXL, a protein that makes cancers and their environment highly aggressive and which is associated with poorer outcomes across many cancers

Diversified pipeline, lead drug is tested in several indications of high unmet medical need and large market potential

Promising efficacy with sustained treatment benefit and confirmed favourable safety

Companion diagnostic supported by biomarker tests

BGB324

First-in-class highly selective small molecule AXL inhibitor

Broad phase II clinical programme in NSCLC, TNBC, AML/MDS, melanoma

Pipeline

BGB324

AXL antibody

AXL ADC (partnered)

Immunomodulatory small molecules

OSE:BGBIO

Raised USD 50m in IPO on OSE in April ’17

USD 150m market cap (Jan 9th 2018)

Corporate

35 staff

Headquarters and research in Bergen, Norway; Clinical Trial Management in Oxford, UK

BerGenBio is developing AXL inhibitor drugs to treat aggressive cancers

BGB324 Phase II clinical trials
AXL inhibition as cornerstone for cancer therapy

BGBC008: **NSCLC**			
BGBC007: **TNBC**	BGBIL006: **Melanoma**	BGBIL005: **NSCLC**	
BGBIL006: **Melanoma**	BGBC004: **NSCLC**	BGBC003: **AML**	BGBC003: **AML/MDS**
+ checkpoint inhibitors	+ targeted therapy	+ chemotherapy	monotherapy

BGB324 foundation therapy

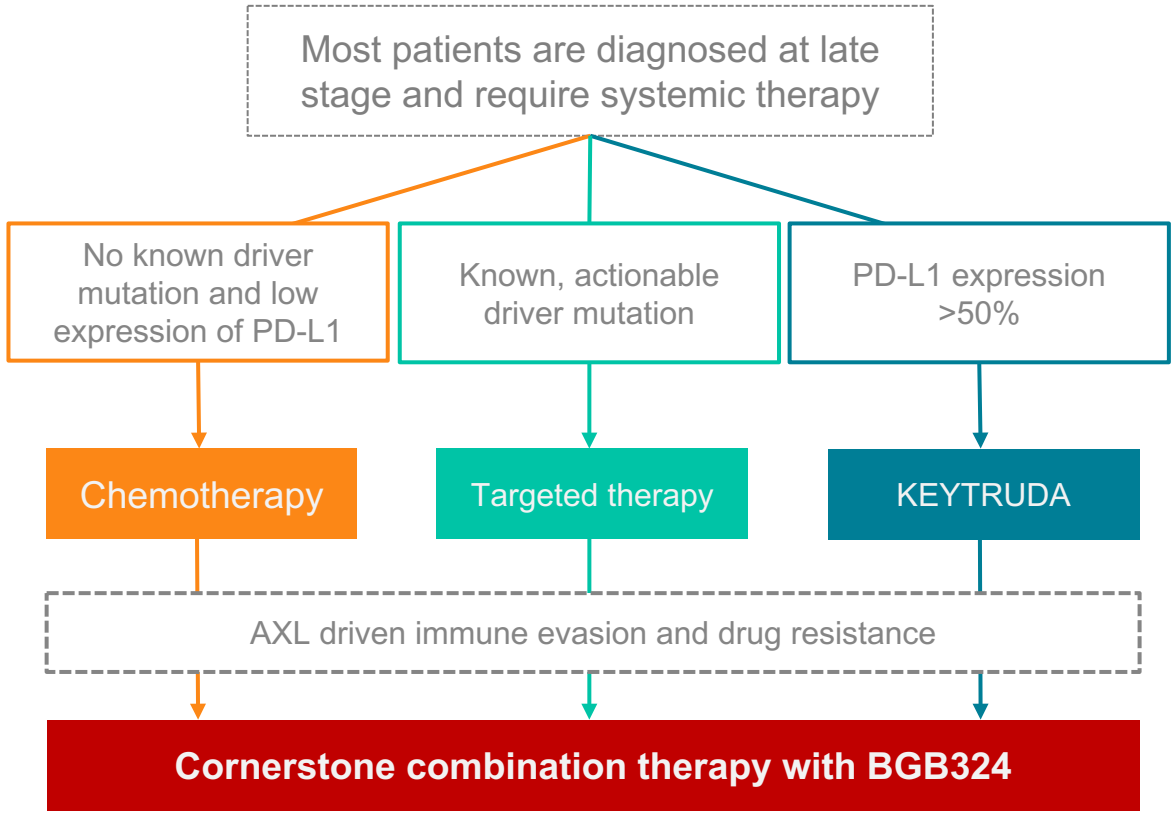
Recent highlights from clinical studies:

- BGBC004 & BGBIL005 – Lung cancer
- BGBC003 – Leukaemia

BGBC004 + BGBIL005

AXL inhibition as cornerstone of therapy in NSCLC

Potential for BGB324 to become a cornerstone therapy for NSCLC

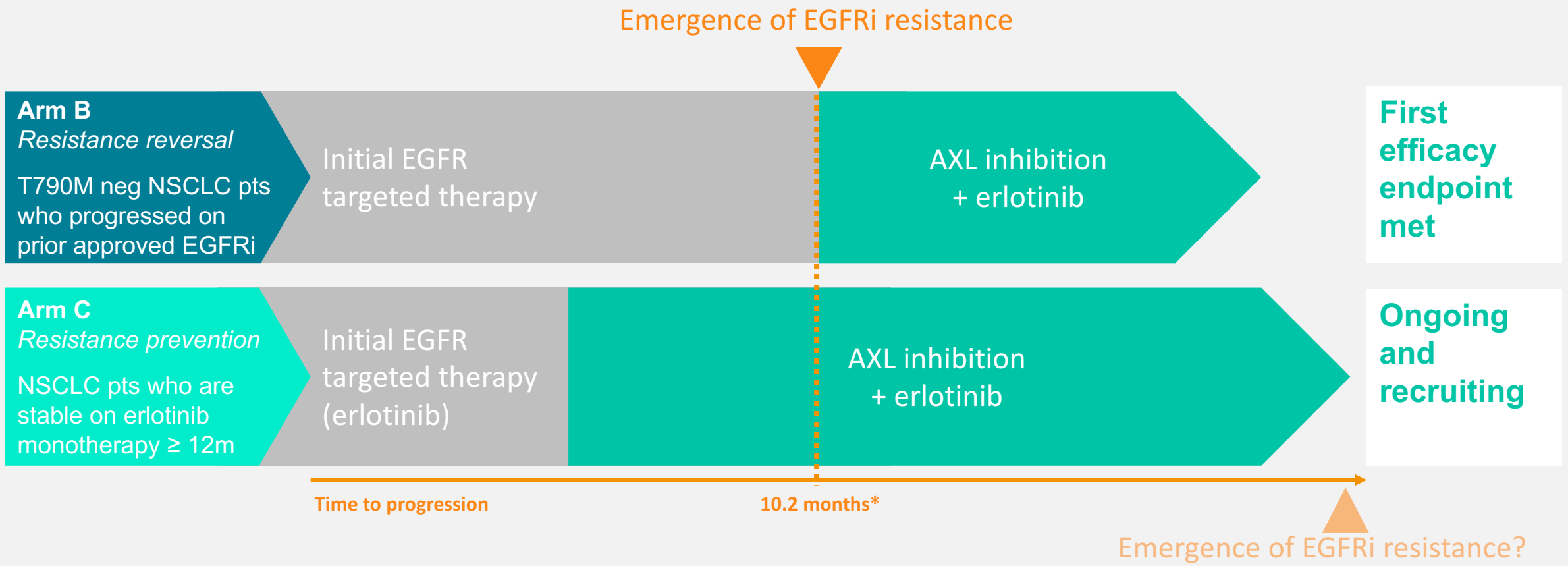


- Lung cancer is the most frequent cause of cancer-related death in developed countries
- Strategy to position BGB324 as the cornerstone of treatment for NSCLC by combining with standard of care therapies

BGBC004

NSCLC patients with EGFR mutation, reversal and prevention of resistance to EGFR targeted therapy, BGB324 + erlotinib

Designed to evaluate the potential of BGB324 to reverse and prevent acquired resistance to EGFR targeted therapy: Reversal setting (arm B) successfully completed first stage



* FLAURA trial comparator arm, SoC erlotinib or gefitinib

BGBC004: Phase II trial in NSCLC of BGB324 with TARCEVA (erlotinib)

BGBC004 Phase II – NSCLC EGFR-mutation driven

Stage IIIb or IV disease EGFR mutation positive

up to 66 pts

Dose escalation (completed) & expansion (ongoing)

Arm A: dose finding

Arm B: 2nd line
BGB324 200mg/d
erlotinib

Arm C: 1st line
BGB324 200mg/d
erlotinib

Safety

Efficacy, PK, biomarkers

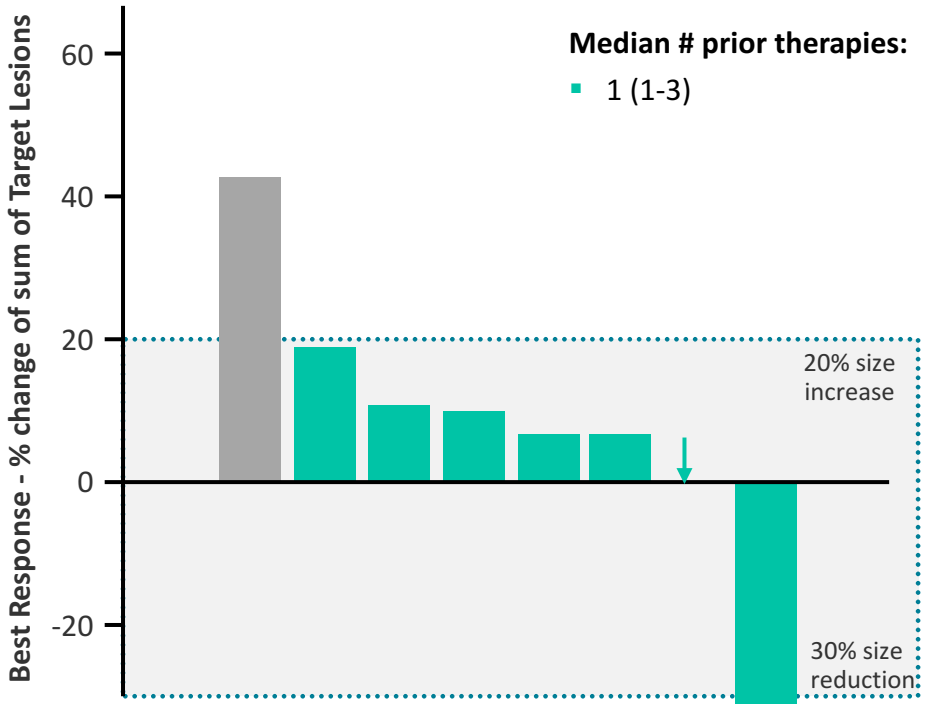
Expected readout

Initial read-out expected 2H 2018

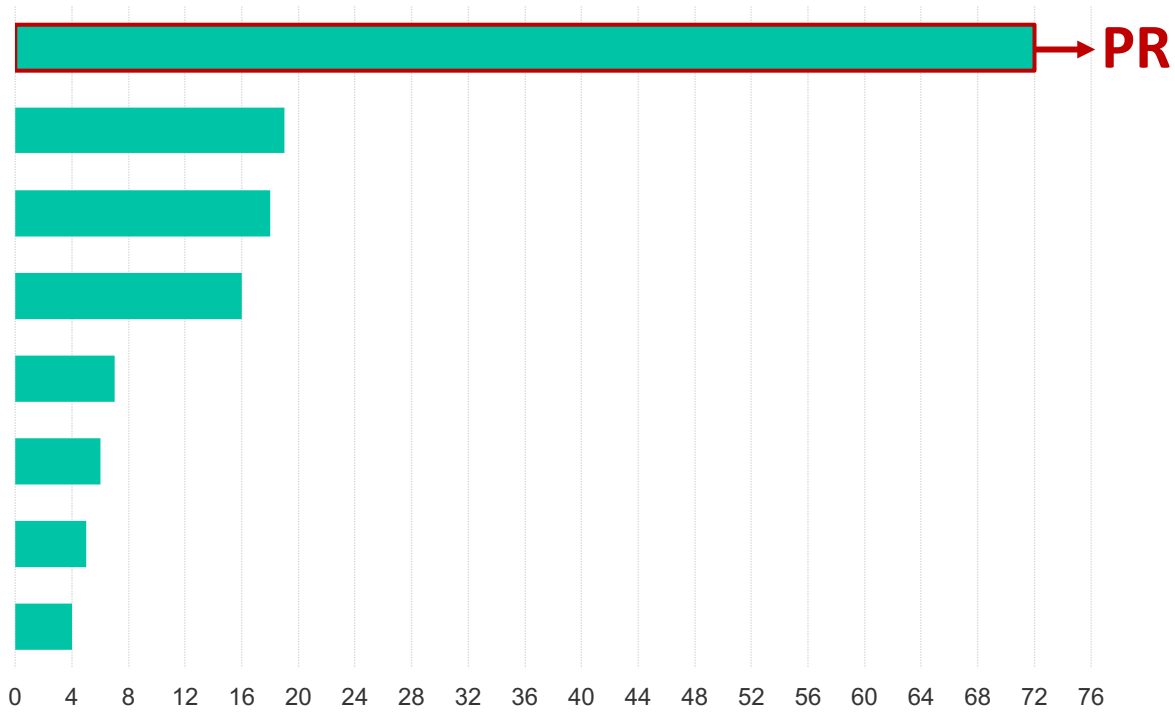
Source: NCT02424617

BGBC004: Interim clinical data Phase II trial in NSCLC of BGB324 with TARCEVA (erlotinib) – arm A – dose finding

Best response (CT scan every 6 weeks)



Duration of treatment (weeks)



Clinical benefit
- 3 SD > 4 months
- 1 stable disease

One patient ongoing > 20 months

Well tolerated

Recommended Phase II dose

Status October 2017

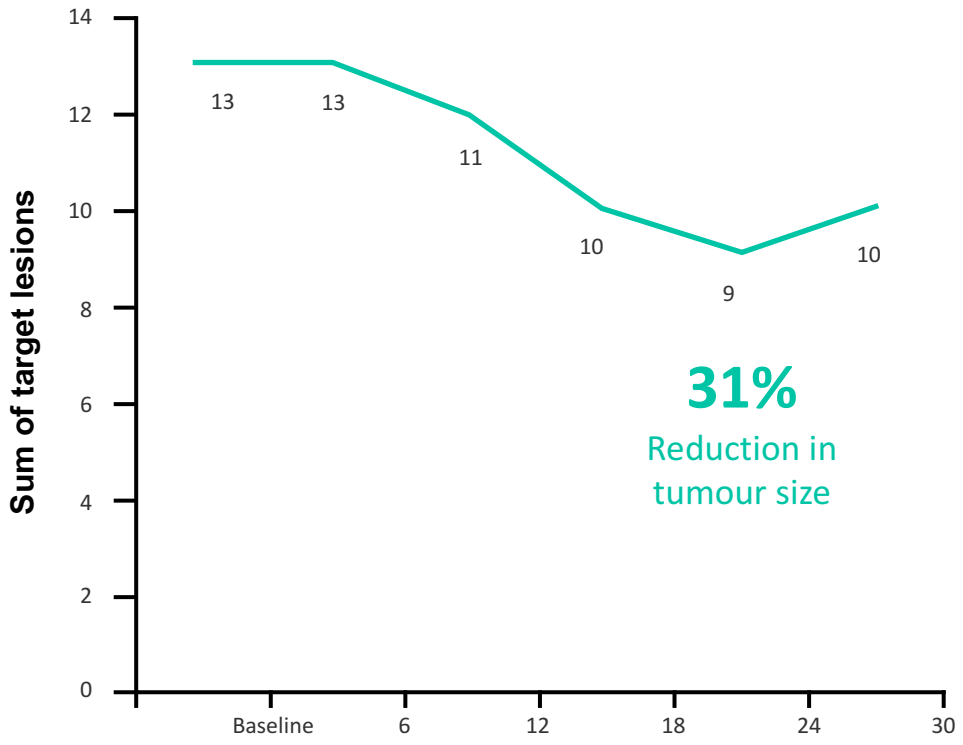
Source: Byers *et al* World Conference on Lung Cancer

BGBC004: Interim clinical data Phase II trial in NSCLC of BGB324 with TARCEVA (erlotinib) – arm A

Pt 311-210 characteristics

Age, ethnicity & sex	68 year old white female
ECOG	1
Histologic diagnosis	Adenocarcinoma of the lung
Stage **at initial diagnosis** **at screening**	 IV IV
Sites	Lung, bone
Mutations	EGFR: exon 21 L858R substitution mutation
Previous lines of therapy **Feb 2015 – Oct 2015** **Oct 2015 – Feb 2016**	 DACOMITINIB ERLOTINIB
Current status	Ongoing, C24

Pt 311-210 response assessment (weeks)

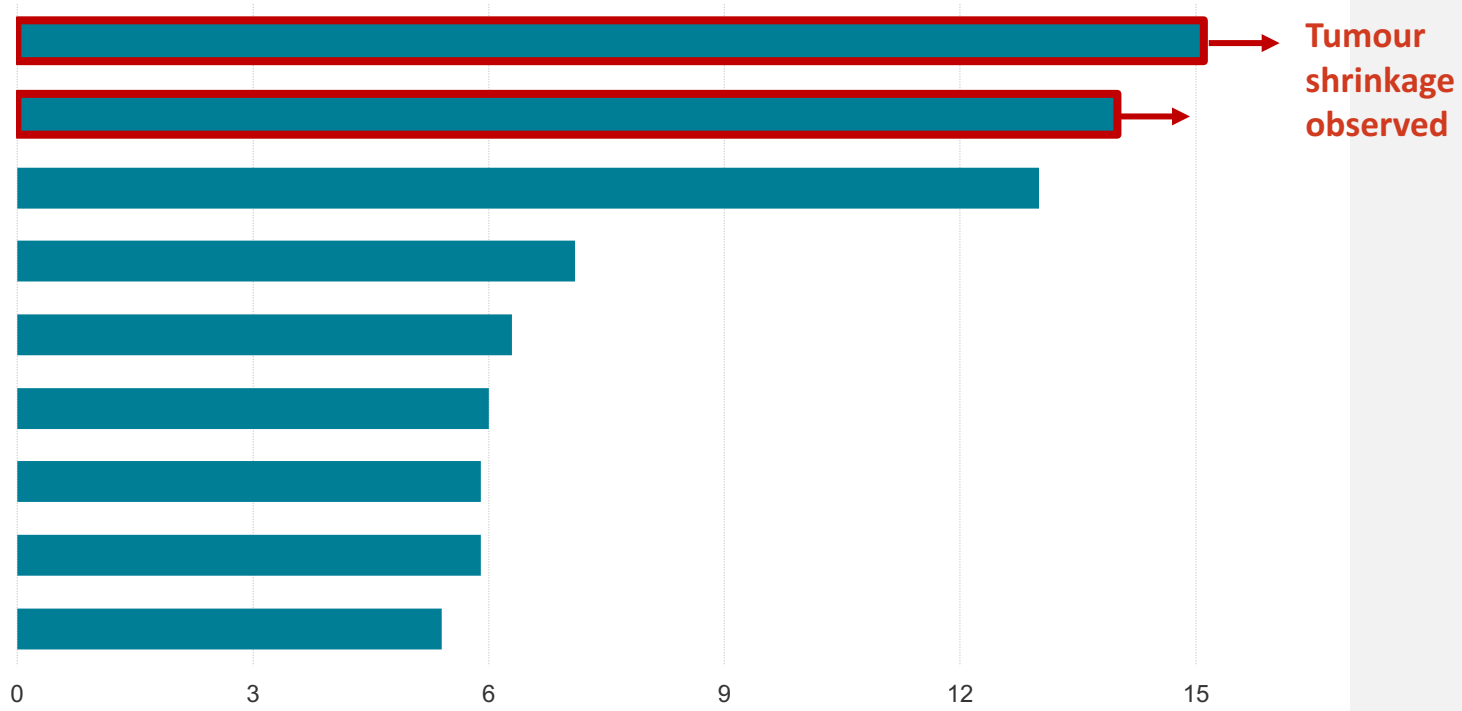


Status October 2017

Source: Byers *et al* World Conference on Lung Cancer

BGBC004: arm B, resistance reversal

Duration of treatment (weeks)



Clinical benefit

- 2 SD > 4 cycles
- 3 SD at 6 wks

Status January 2018

Arm B patient population

- **T790M negative**
- **Median 3 lines (2 – 12)** prior therapy
- **Progressed on 1st line EGFR TKI therapy (erlotinib, afatinib, gefitinib)**
- 5 of 9 pts are Asian, 6 females (typical EGFRm population)

Source: Byers *et al* World Conference on Lung Cancer

BGBIL005

NSCLC patients,
last line setting
BGB324 + chemo (docetaxel)

Docetaxel is main treatment option in NSCLC after chemo failure and as last line after failure of chemo, targeted and/or IO

Recent results in recurrent NSCLC (chemo failure) with docetaxel

	Study	Intervention	ORR
Single agent	CheckMate 057: Borghaei *et al*[1] 582 patients randomised Pt chemo failures	Nivolumab vs Docetaxel	**19%** **12%**
	OAK trial: Marinis *et al*[2] 850 patients randomised Pt chemo failures	Atezo vs Docetaxel	**14%** **14%**
	KEYNOTE 010[3] ≥ 1% PDL1	Pembro Docetaxel	**19%** **9%**
Combination	*BGBIL005*	Bemcentinib (BGB324) + docetaxel	**33%**
	Levvy *et al*[4] 95 patients randomised	Docetaxel + PX-866 (PI3K inhibitor) vs Docetaxel alone	**6%** **0%**
	Ramlau *et al*[5] 913 patients randomised	Docetaxel + Aflibercept (anti-VEGF) vs Docetaxel alone	**23%** **9%**

(1) Borghaei, NEJM (2015) (2) Marinis ESMO 2017(3) Keytruda label (4) Levy, JTO 2014 (5) Ramlau JCO 2012

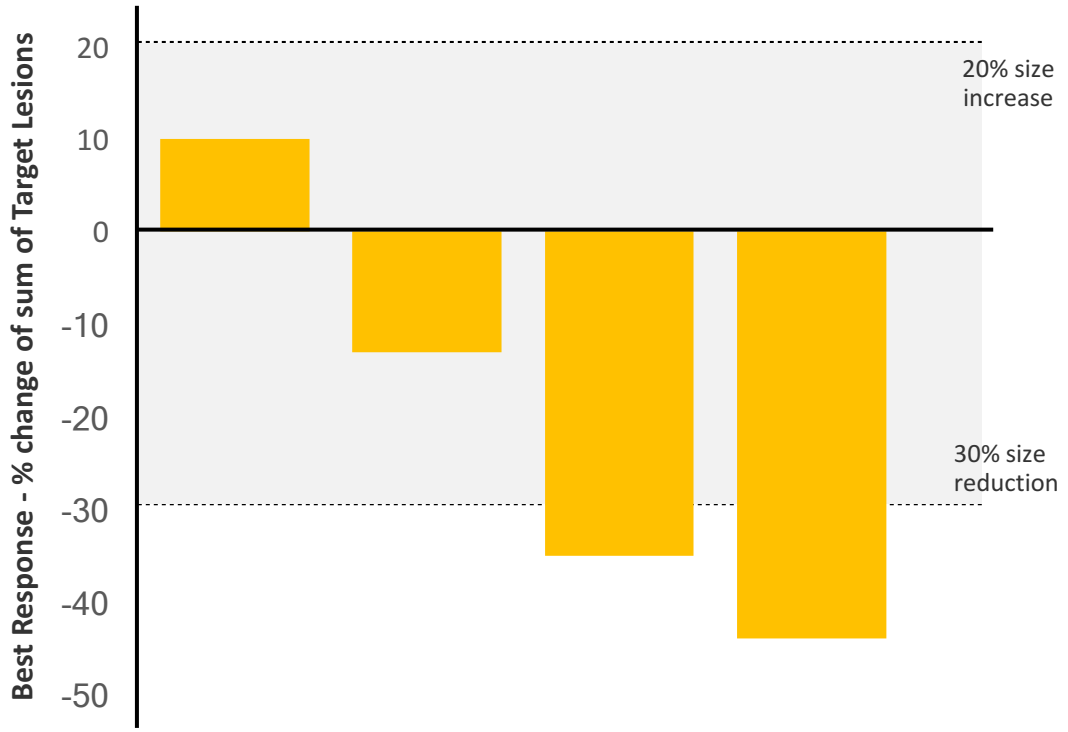
85,000* NSCLC patients receive docetaxel in later line
70% mortality within 1y

BGBIL005 – patient population

Heavily pre-treated patient population:

- All failed at least 1 line of chemo
- Most received prior immunotherapy without sustained benefit
- Most patients are metastatic
- No more treatment options remain

Best response (CT scan every 6 weeks)



* Per annum company estimate based on prevalence and prescription information

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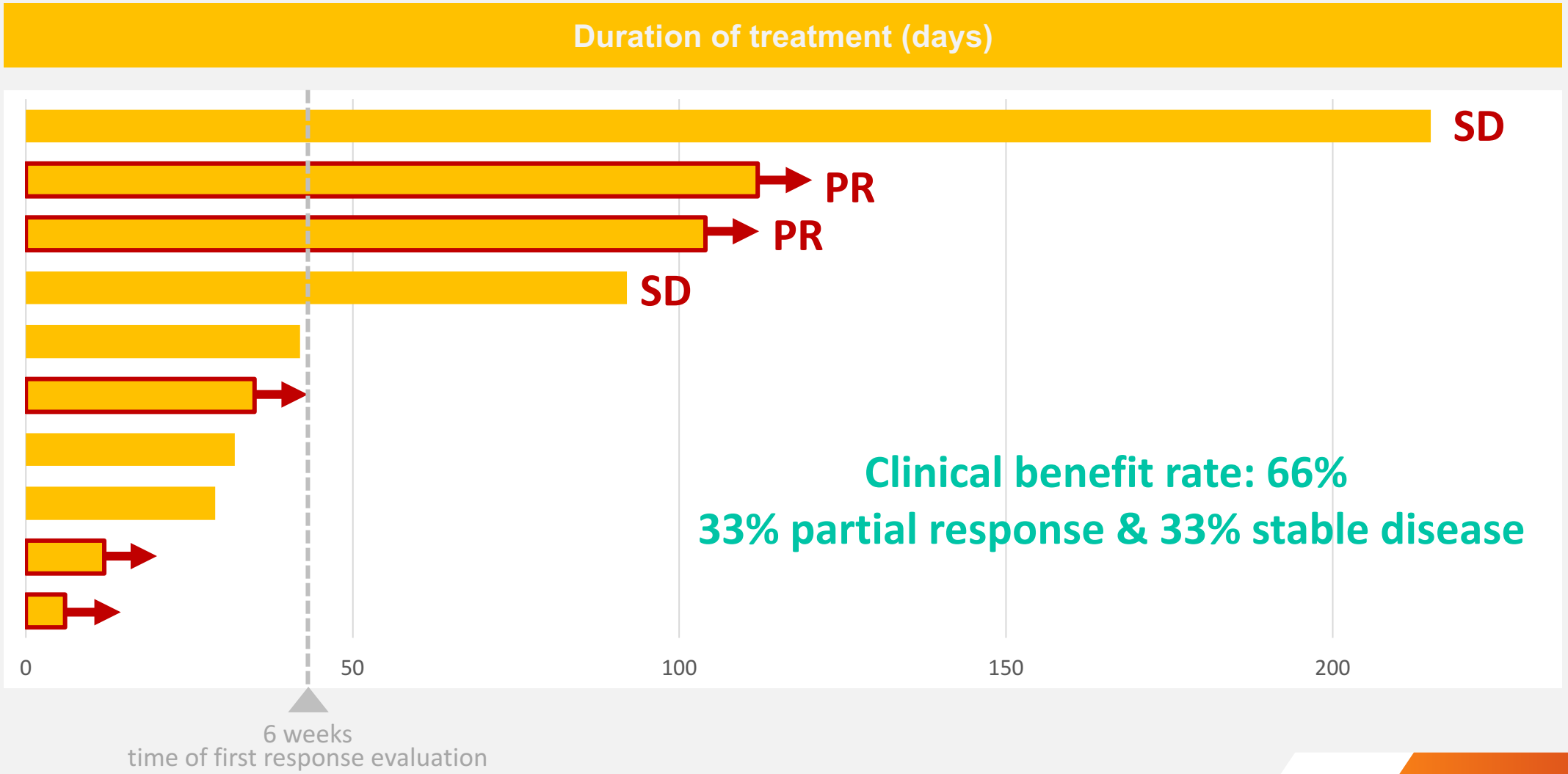
BGB324 + docetaxel in NSCLC patients (last line setting)



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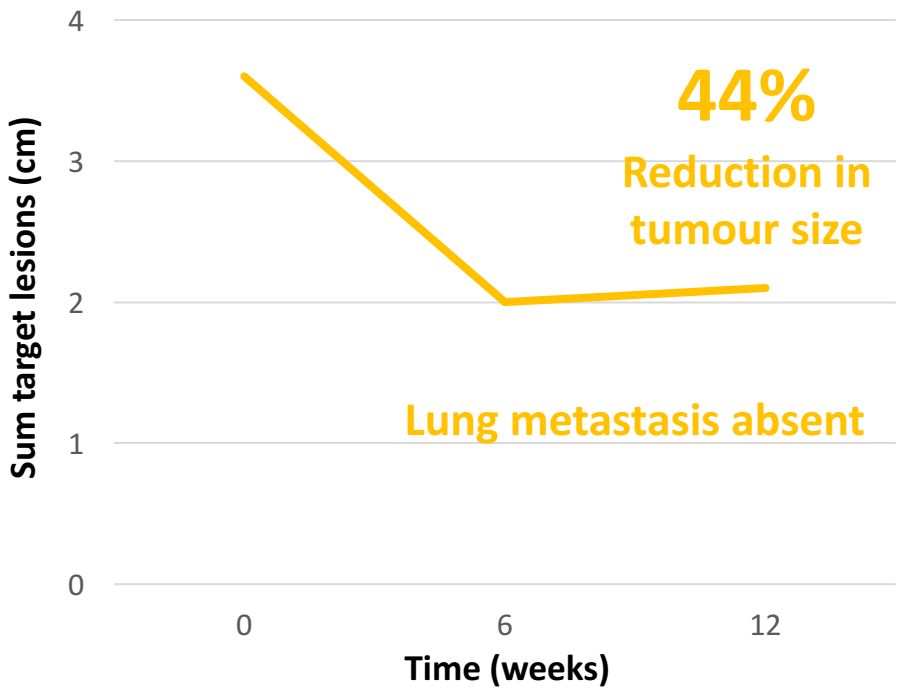
BGBIL005, Patient case # 004:
PR on BGB324 + docetaxel after failure on chemo and IO

Pt 004 characteristics

Age, ethnicity & sex	63 year old Caucasian female
Histologic diagnosis	NSCLC
Stage	IV
Sites	Lung, lymph, lung metastasis
Mutations	None (EGFR wt, ALK negative)
Previous lines of therapy	CARBOPLATIN/PACLITAXEL CARBOPLATIN/PEMETREXED PEMBROLIZUMAB
Current status	Ongoing, C5

Pt 311-210 response assessment (weeks)



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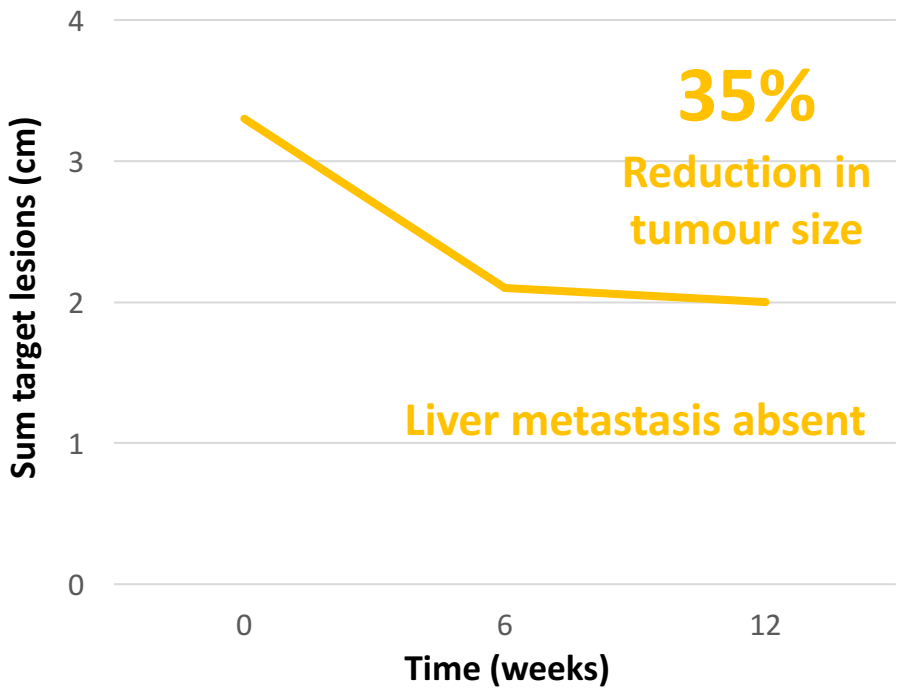
BGBIL005, Patient case # 006:
PR on BGB324 + docetaxel after failure on chemo and IO

Pt 006 characteristics

Age, ethnicity & sex	53 year old Asian male
Histologic diagnosis	NSCLC
Stage	IV
Sites	Lung, lymph, liver, brain
Mutations	None (EGFR wt, ALK negative)
Previous lines of therapy	CISPLATIN/PEMETREXED CISPLATIN VINORELBINE NIVOLUMAB
Current status	Ongoing, C5

Pt 311-210 response assessment (weeks)



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BGBIL005: Phase I/II trial in NSCLC, BGB324 with docetaxel – remains ongoing.

BGBIL005 Phase I/II – NSCLC (2nd line – progressed/treatment-refractory disease) – *Investigator-sponsored study*

Advanced NSCLC, exhausted all treatment options

up to 30 pts
any prior treatment

3+3 dose escalation & expansion

Single arm

BGB324 100 mg/d
Docetaxel 60 mg/m^2

Safety

ORR, PFS, OS, PK, biomarker assessments

Expected readout

Initial read-out expected 2H 2018

Sponsor Investigator: Dr David Gerber, UTSW Dallas

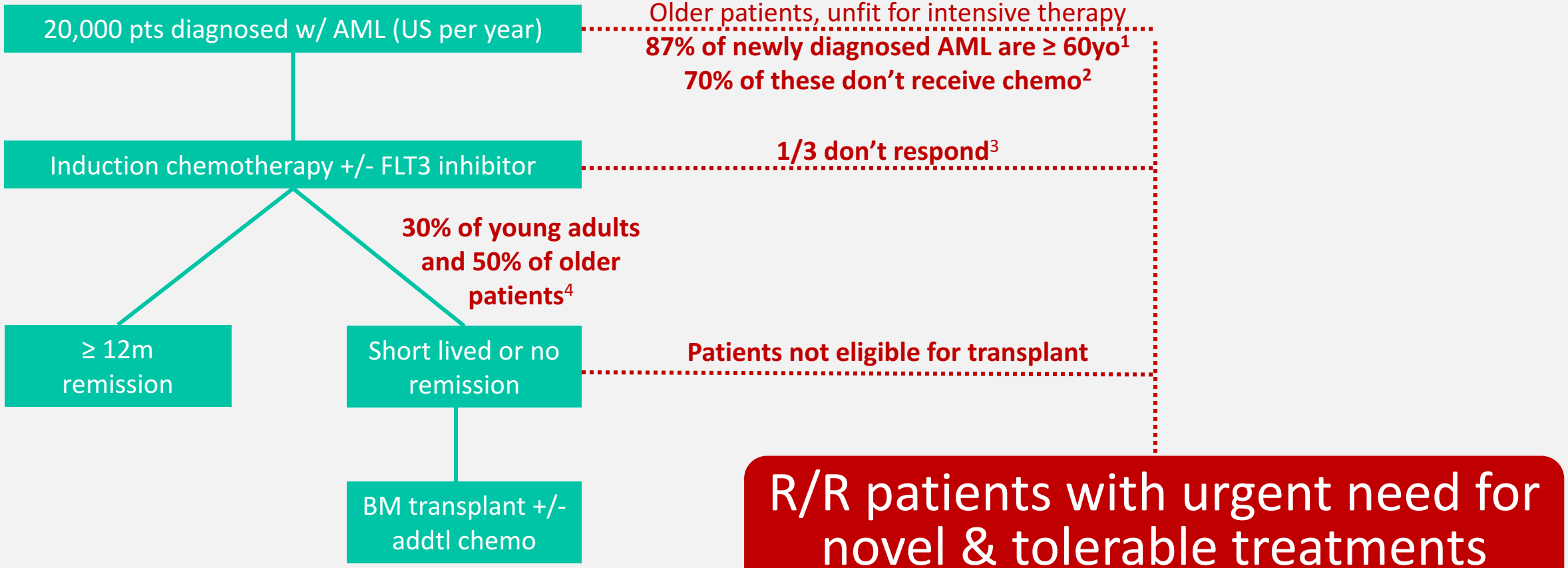
"The vast majority of my lung cancer patients progress onto chemotherapy, combining this with BGB324 may significantly improve the performance of the chemo and could lead to meaningful disease modification in some patients."

Source: NCT02922777

BGBC003

Relapsed & refractory AML and high risk MDS

Relapsed/refractory AML & MDS – Blood cancer, difficult to treat malignancies, predominantly elderly frail patient population.



MDS: Myelodysplastic syndrome

Source: (1) Juliusson, Blood (2009) (2) Erba, Leukemia Research (2015) (3) cancer.org (4) uotodate.com

Clinical Trial data for R/R AML patients from ASH December 2017

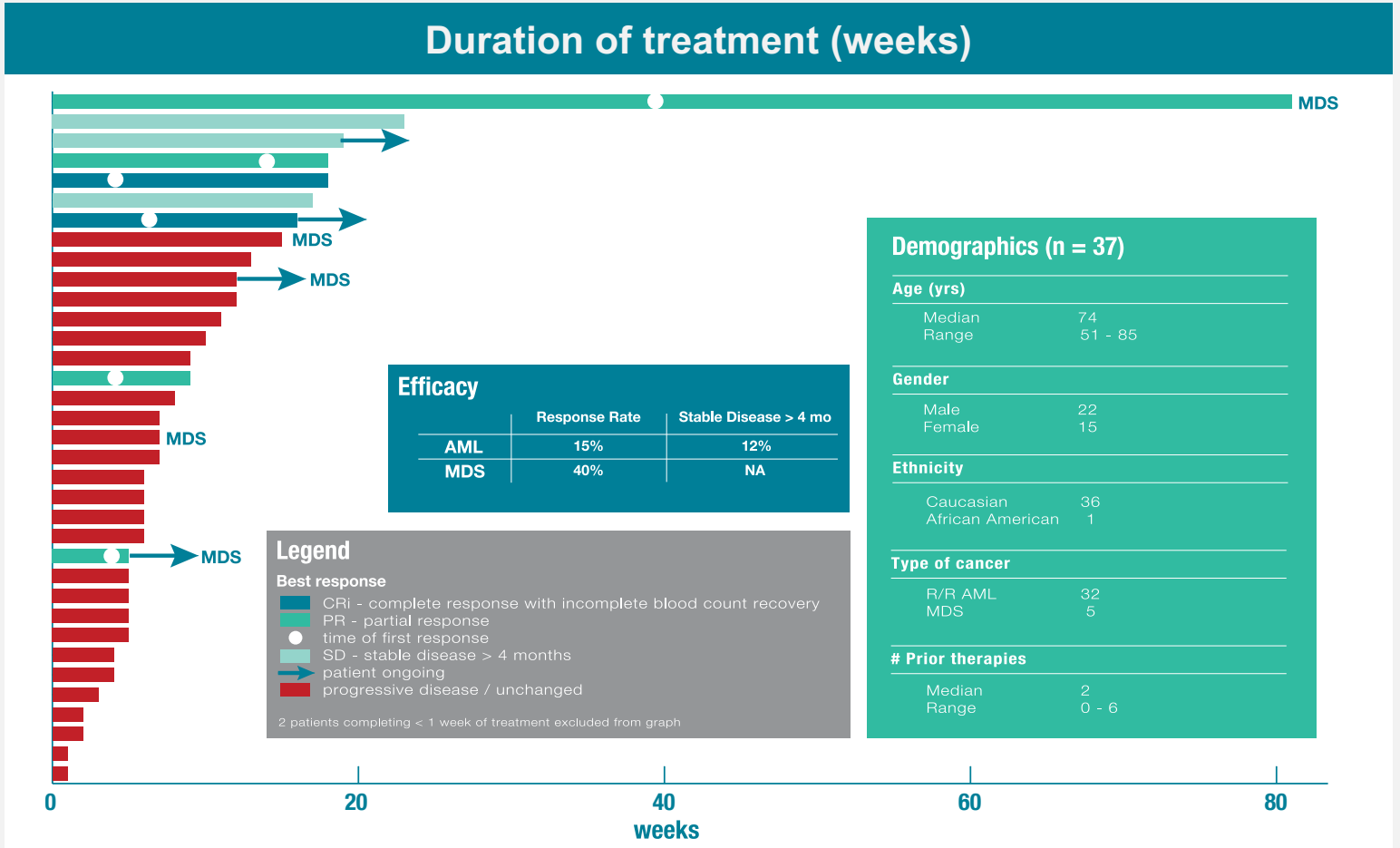
American Society of Hematology
Helping hematologists conquer blood diseases worldwide

	Study	Intervention	ORR
Single agent	***BerGenBio*** *37 patients*	*BGB324* *all comers, elderly R/R patients*	***19%***
	Pratz *et al*[1] 31 patients	TAK-659 investigational FLT-3 and SYK inhibitor	**9%**
	Daver *et al*[4] 51 patients	FLX925 Dual FLT3 and CDK4/6	**0%**
	Dawson et al[6] 46 patients	GSK525762 BET inhibitor	**11%**
	DiNardo *et al*[5] 258 patients – ***selected for mIDH1 mutation***	Ivosidenib (AG-120) mutant IDH1 (mIDH1) inhibitor	**30%**
Combination	Goldberg *et al*[2] 24 patients	Venetoclax* + hypomethylating agent (HMA) or low dose cytarabine (LDAC)	**28%**
	Rausch *et al*[3] 27 patients	Venetoclax + HMA or LDAC	**22%**

***Venetoclax + LDAC received breakthrough designation in 1st line AML (July 2017)**

(1) ASH 2017 abstract 2622 (2) ASH 2017 abstract 1353 (3) ASH 2017 abstract 1356 (4) ASH 2017 abstract 1343 (5) ASH 2017 abstract 725 (6) ASH 2017 abstract 1377

Superior early monotherapy efficacy with favourable safety in R/R AML & high risk MDS reported at ASH 2017



19% Response Rate (CRi + PR)

- 2 CRi
- 5 PRs

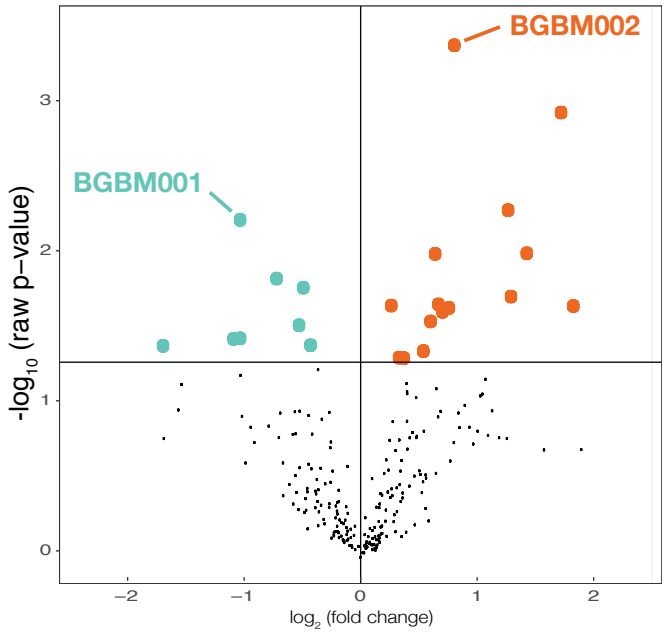
An additional 7 patients were stable > 4 months

Well tolerated

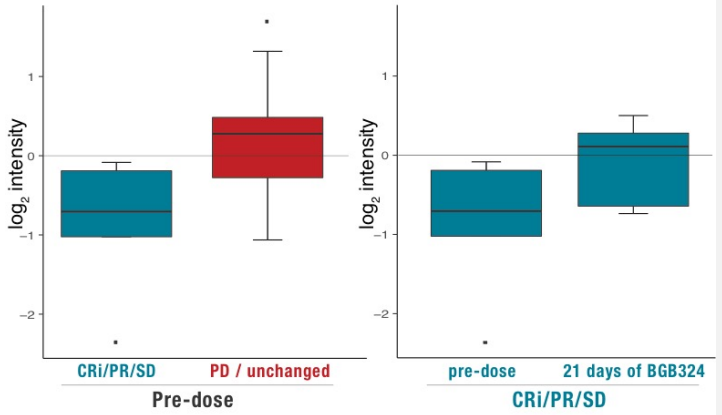
Correlation with predictive biomarker candidates

BerGenBio AML blood based biomarkers predict patients benefitting from BGB324 therapy

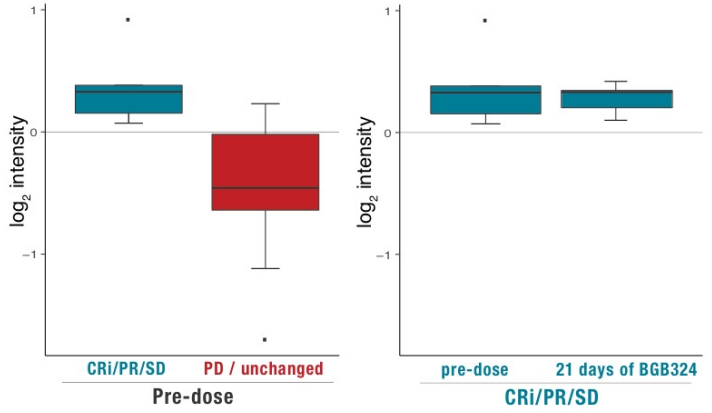
Liquid biopsy in blood plasma: BGBM001 & BGBM002



BGBM001 in blood plasma



BGBM002 in blood plasma



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BGBC003: Phase II trial in AML and MDS – remains ongoing.

BGBC003 Phase II – AML/high risk MDS as monotherapy and in combination with decitabine or azacitidine

Relapsed/refractory AML & high-risk MDS

up to 75 pts

Dose escalation (completed)

AML

2nd line monotherapy

1st line combo
BGB324 + decitabine or azacitidine

MDS

2nd line monotherapy

Safety & efficacy

PK, biomarkers

Expected readout

Initial read-out expected 2H 2018

BGB324 is an AXL inhibitor to target aggressive cancers…

50%

of people will get a form of cancer in their lifetime

90%

of cancer deaths due to aggressive cancer

Treat. Reverse. Stop.

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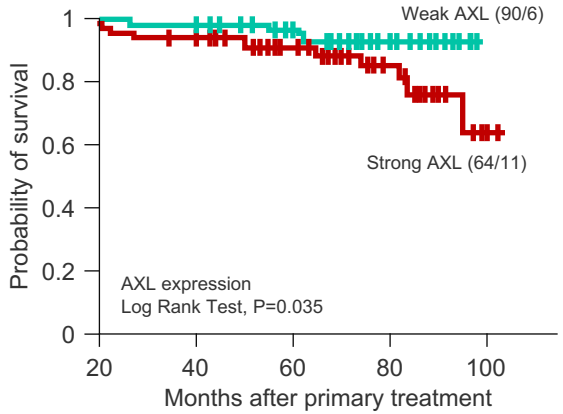
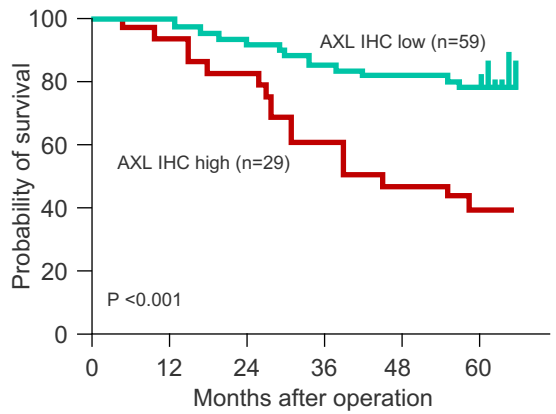
Aggressive cancers

Strong AXL expression correlates with poor survival rate

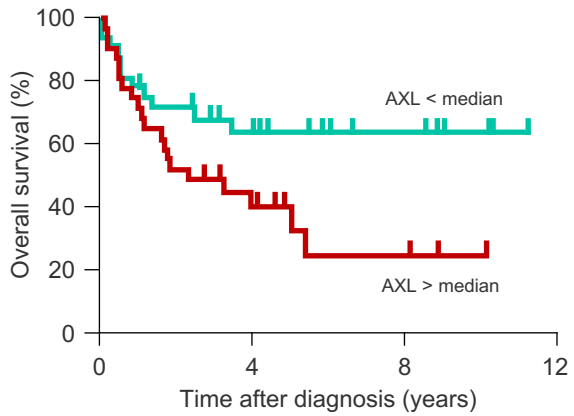
Breast carcinoma[1]



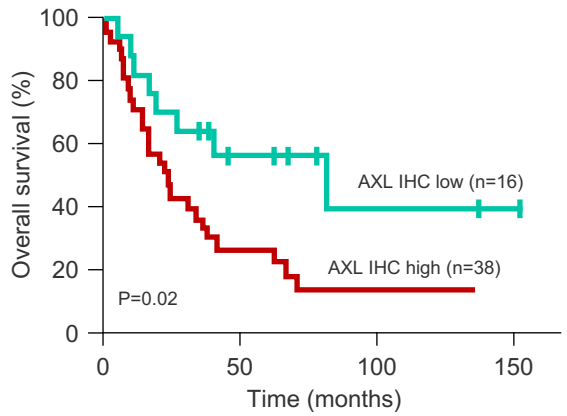
Lung adenocarcinoma (NSCLC)[2]



Acute Myeloid Leukaemia[3]



Pancreatic ductal adenocarcinoma[4]



Broad evidence of AXL linked with poor prognosis[5]

Astrocytic brain tumors	Melanoma
Breast cancer	Mesothelioma
Gallbladder cancer	NSCLC
GI	Pancreatic cancer
• Colon cancer	Sarcomas
• Esophageal cancer	• Ewing Sarcoma
• Gastric cancer	• Kaposis sarcoma
Gynaecological	• Liposarcoma
• Ovarian cancer	• Osteosarcoma
• Uterine cancer	Skin SCC
HCC	Thyroid cancer
HNC	Urological
Haematological	• Bladder cancer
• AML	• Prostate cancer
• CLL	• RCC
• CML	

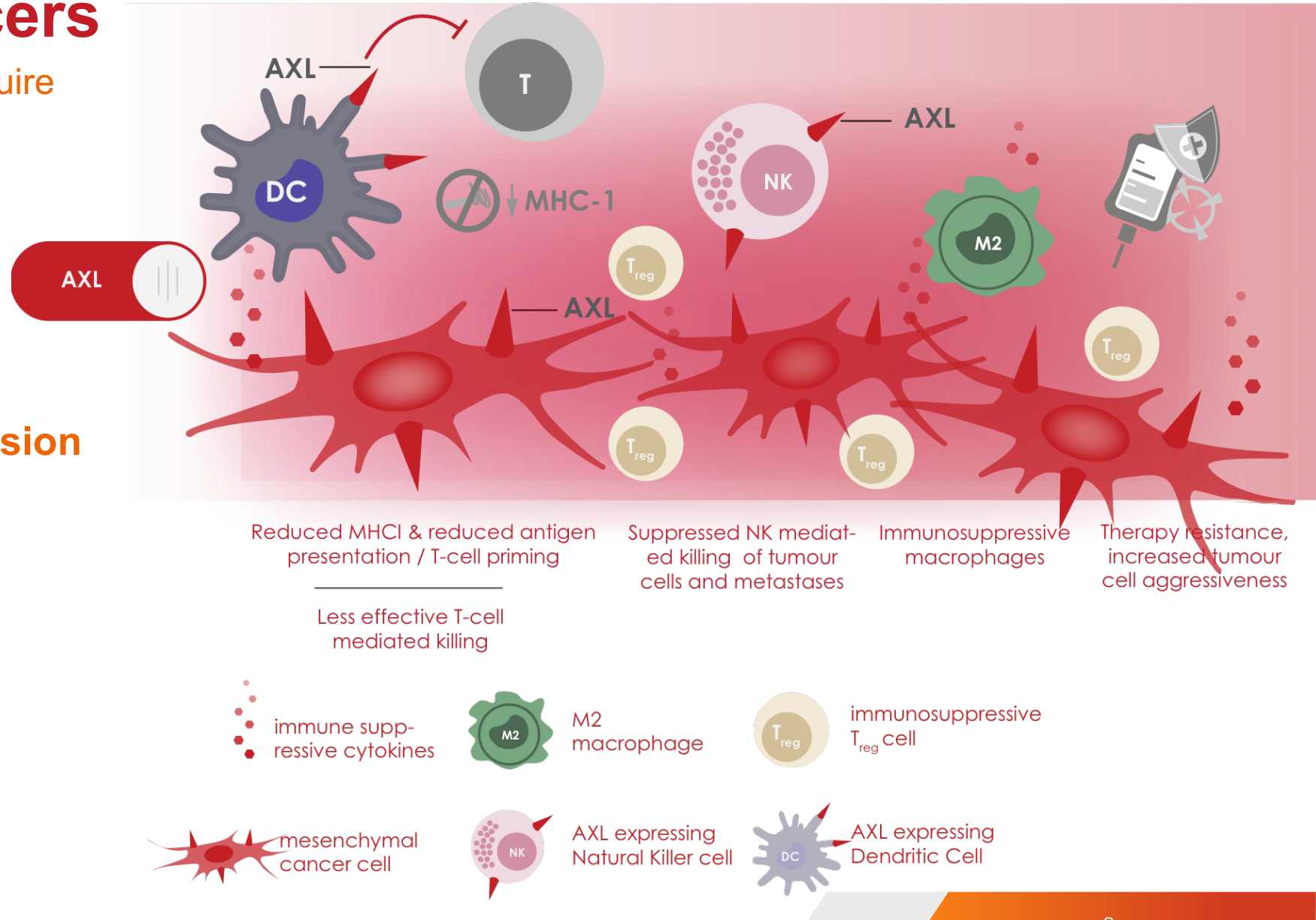
1 Gjerdrum, 2010; 2 Ishikawa, 2012; 3 Ben-Battala, 2013; 4 Song, 2010, 5 supported by > 100 publications

Aggressive cancers

evade the immune system, acquire drug resistance and spread

AXL is a key regulator of aggressive cancers driving:

- **Innate immune suppression**
- **Therapy resistance**
- **Cancer spread**



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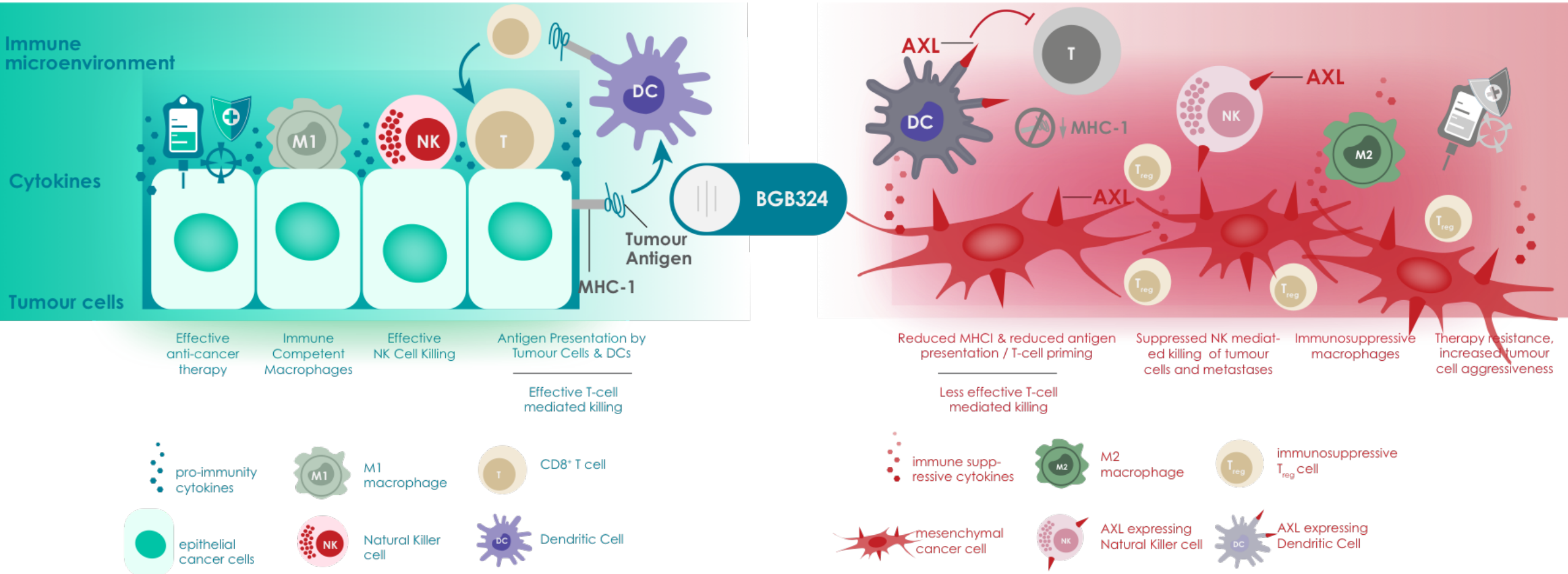
BGB324: selective AXL inhibitor, restores sensitivity to immune cell attack and therapy, prevents spread



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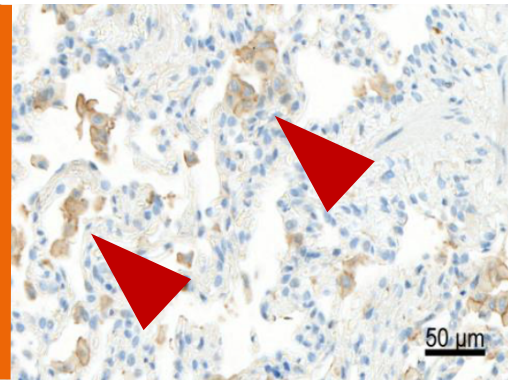
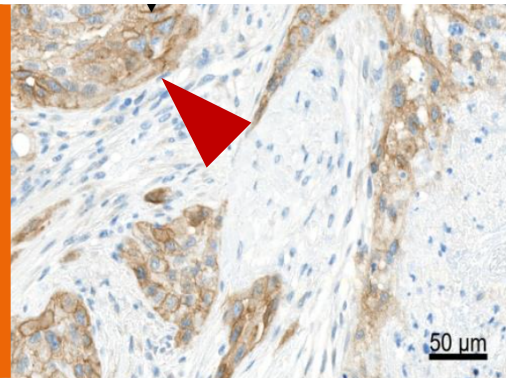
BGB324 targets immunosuppression and therapy resistance

Lung cancer patient samples

Axl is expressed on immune cells in the tumour*

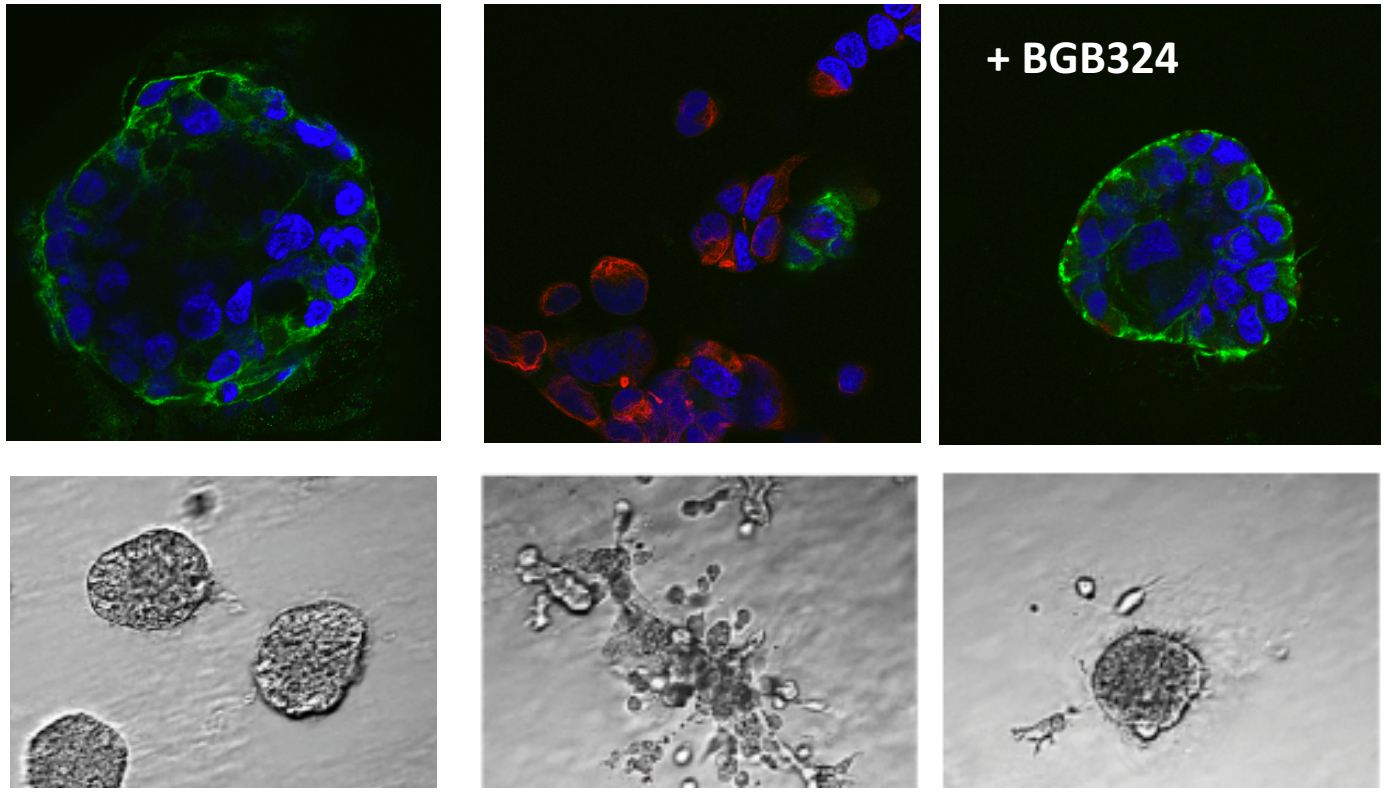


Axl is expressed on tumour cells*



Non aggressive

AXL programme induced



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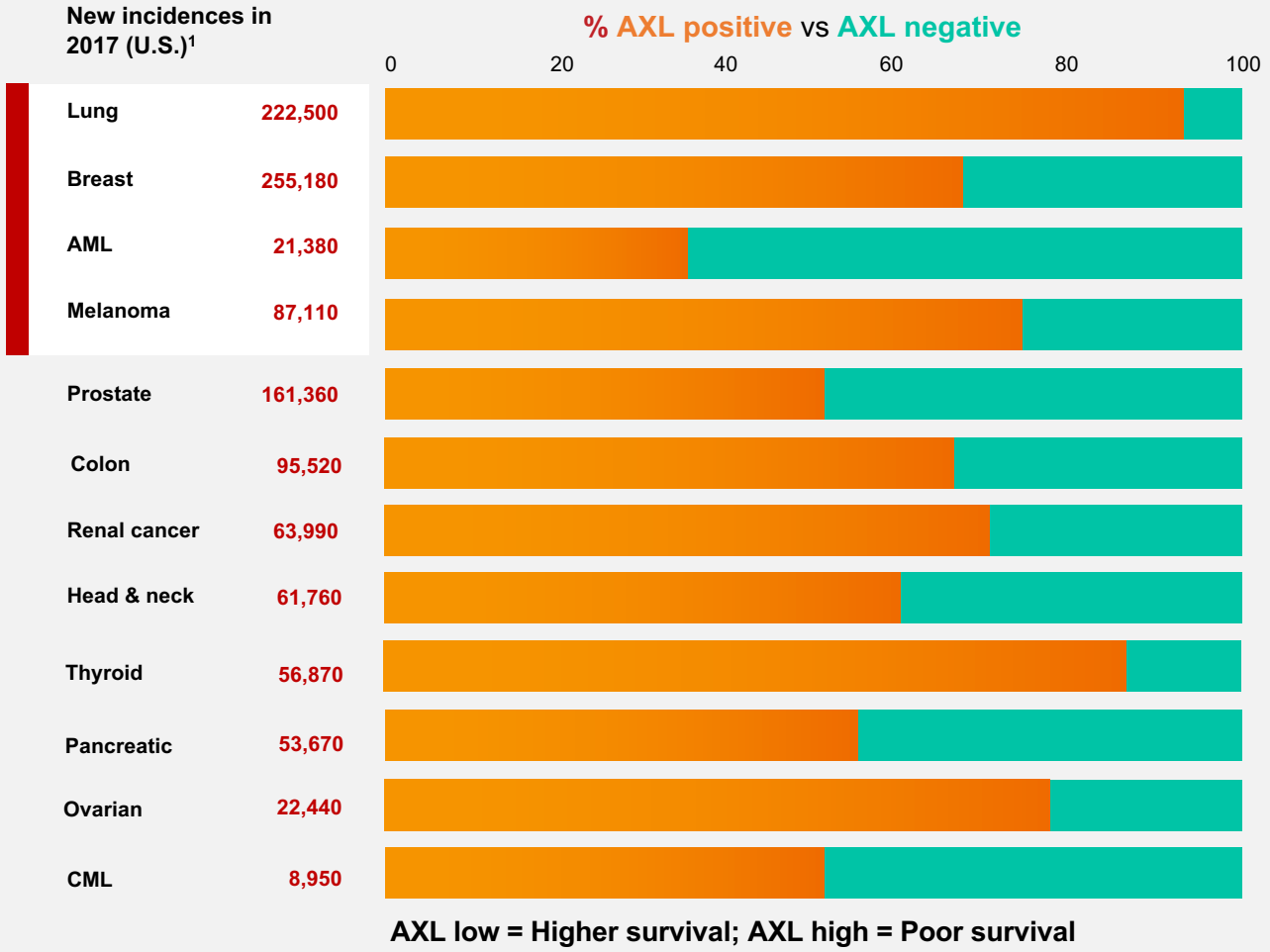
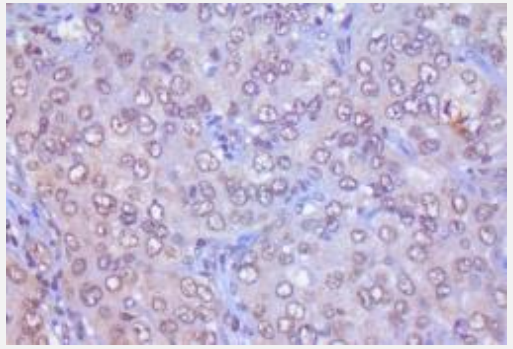
Which cancers are we targeting

Most common tumours express high AXL levels

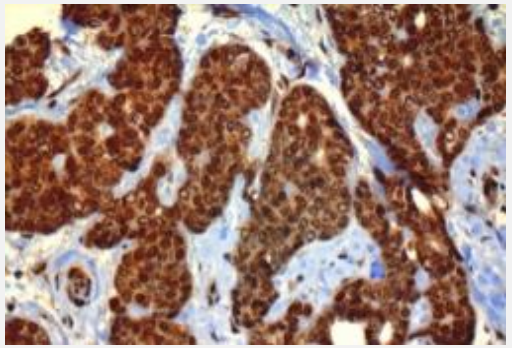


AXL low = Higher survival; AXL high = Poor survival

Low Axl expression[2]



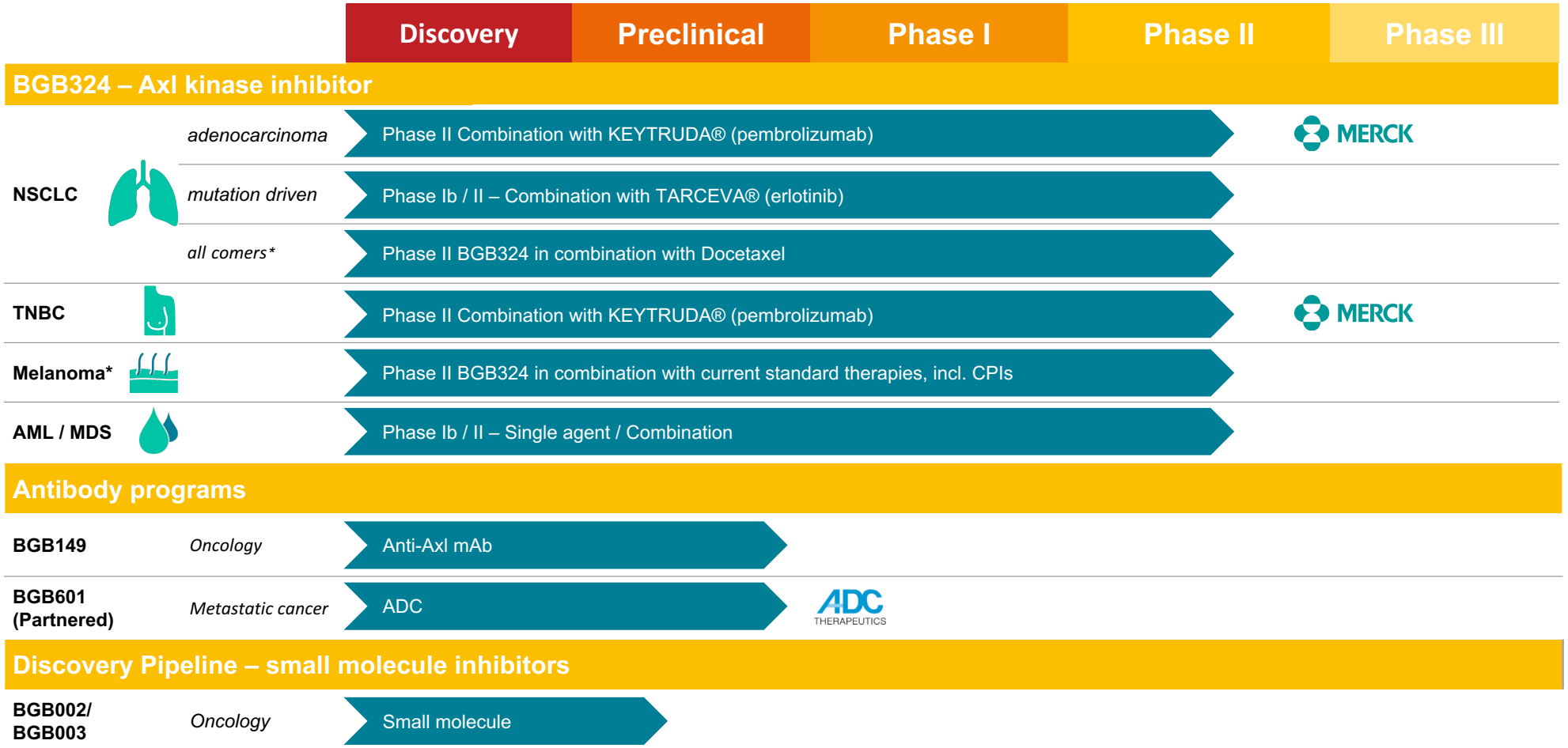
High Axl expression[2]



Companion diagnostic in development to identify AXL positive patients

(1) SEER (2) Gjerdrum 2010

Advancing a broad clinical development pipeline



Patients:

>350

50

sites in Europe and North America.

Key read-outs:

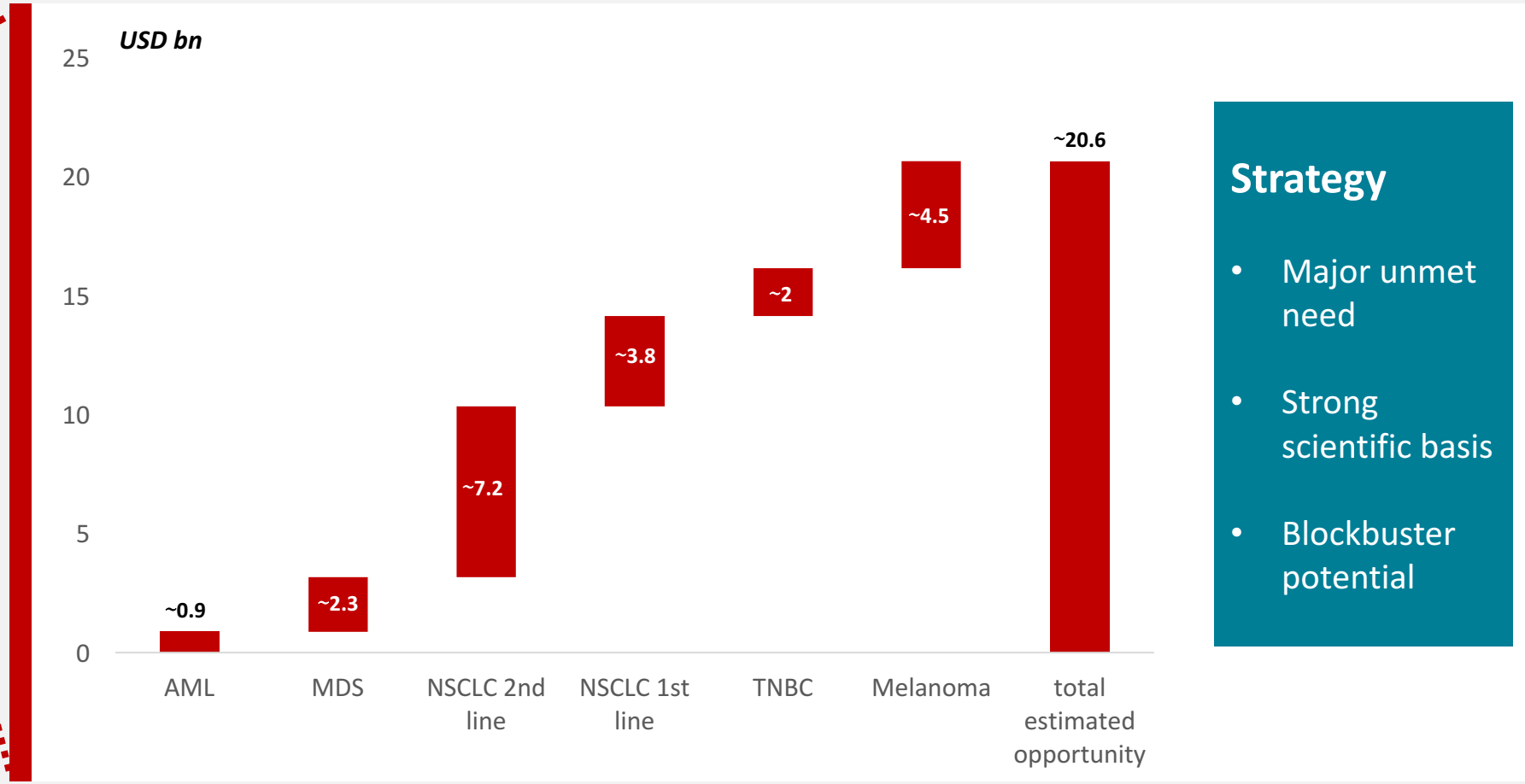
2018

*Investigator-sponsored trials

Targeting cancers with an addressable market of > $20bn

Most common tumours express high AXL levels

New incidences in 2017 (U.S.)[1]	
Lung	222,500
Breast	255,180
AML	21,380
Melanoma	87,110
Prostate	161,360
Colon	95,520
Renal cancer	63,990
Head & neck	61,760
Thyroid	56,870
Pancreatic	53,670
Ovarian	22,440
CML	8,950



Strategy

- Major unmet need
- Strong scientific basis
- Blockbuster potential

(1) SEER

And…it's a simple pill taken once a day

BGB324 clinical development strategy: AXL inhibition as cornerstone for cancer therapy

Last line, heavily pre-treated

R/R AML
19% RR
11% SD > 4mo

High risk MDS
40% RR

NSCLC
25% 1-year PFS

+ checkpoint inhibitors

+ targeted therapy

+ chemotherapy

monotherapy

BGB324 foundation therapy

BGB324 clinical development strategy: AXL inhibition as cornerstone for cancer therapy

Last line, stage IV metastatic NSCLC, heavily pre-treated:

- 66% CBR
- 2 Partial Responses
- Durable response (> 10 cycles)
- Favourable safety

+ checkpoint inhibitors

+ targeted therapy

+ chemotherapy

monotherapy

BGB324 foundation therapy

BGB324 clinical development strategy: AXL inhibition as cornerstone for cancer therapy

Last line, stage IV metastatic NSCLC, EGFR+, heavily pre-treated:

- 50% CBR
- Including 1 PR
- One patient ongoing > 21 months

+ checkpoint inhibitors

+ targeted therapy

+ chemotherapy

monotherapy

BGB324 foundation therapy

BGB324 clinical development strategy: AXL inhibition as cornerstone for cancer therapy

First line metastatic melanoma:

- Favourable safety

+ checkpoint inhibitors

+ targeted therapy

+ chemotherapy

monotherapy

BGB324 foundation therapy

BGB324 ongoing clinical trials
Reporting interim response & safety data on a regular basis

BGBC008: NSCLC
OPEN & RECRUITING

BGBC007: TNBC
OPEN & RECRUITING

BGBIL006: Melanoma
OPEN & RECRUITING
WORLD MELANOMA '17

+ checkpoint inhibitors

BGBIL006: Melanoma
OPEN & RECRUITING
WORLD MELANOMA '17

BGBC004: NSCLC
OPEN & RECRUITING
WORLD LUNG '17

+ targeted therapy

BGBIL005: NSCLC
OPEN & RECRUITING
WORLD LUNG '17

BGBC003: AML
OPEN & RECRUITING

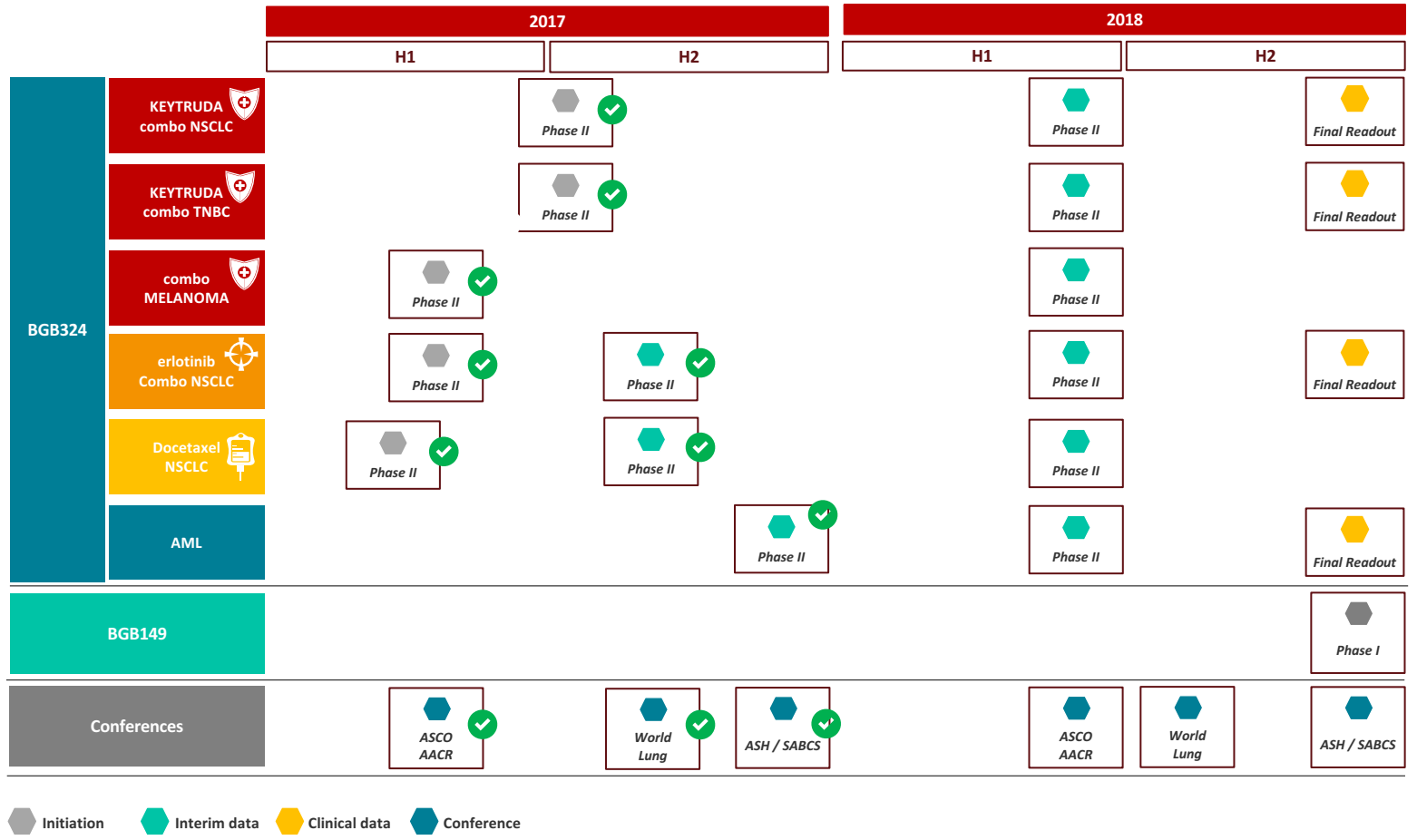
+ chemotherapy

BGBC003:
AML/MDS
ASH '17

monotherapy

BGB324 foundation therapy

Milestones 2017 & 2018



Significant value drivers expected over the next 12 months:

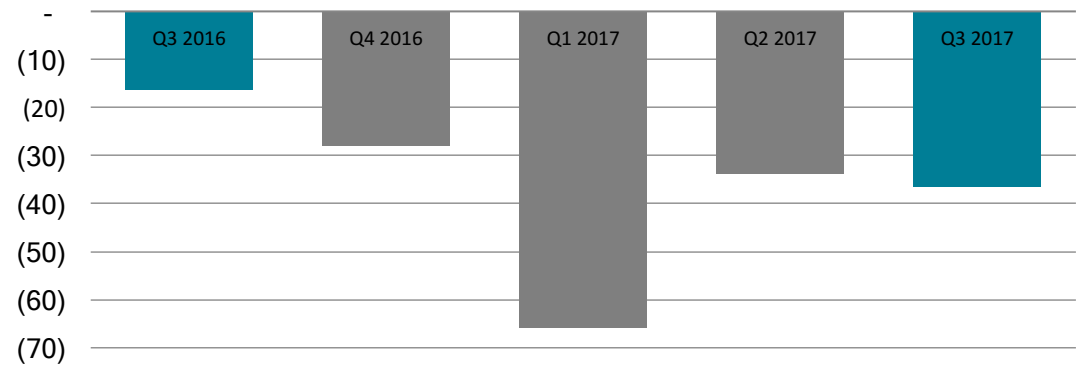
- ✓**Interim clinical data** from 6 ph2 trials **H1'18**
- ✓**Final readout** from 4 phase 2 trials **in H2**
- ✓**Initiation of AXL antibody** BGB149 clinical trials **in H2**

1) Progression of ongoing and start-up of new clinical trials are subject to customary regulatory reviews and approvals

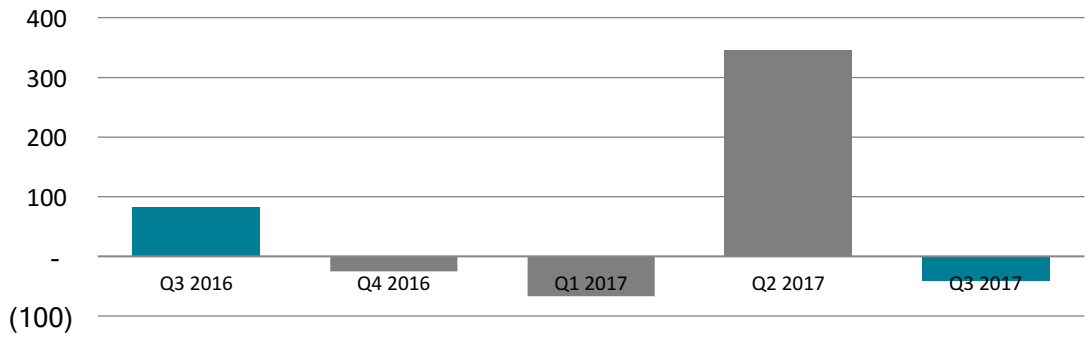
Key financials

Key Figures (NOK million)	Q3 2017	Q3 2016	YTD2017	YTD2016	FY 2016
Operating revenues	-	-	-	-	-
Operating expenses	36.6	16.3	136.2	103.5	131.6
Operating profit (loss)	-36.6	-16.3	-136.2	-103.5	-131.6
Profit (loss) after tax	-35.4	-15.4	-134.6	-101.9	-129.8
Basic and diluted earnings (loss) per share (NOK)	-0.71	-45.64	-3.06	-339.63	-419.68
Net cash flow in the period	-41.1	82.1	237.3	113.2	87.8
Cash position end of period	399.2	187.2	399.2	187.2	161.8

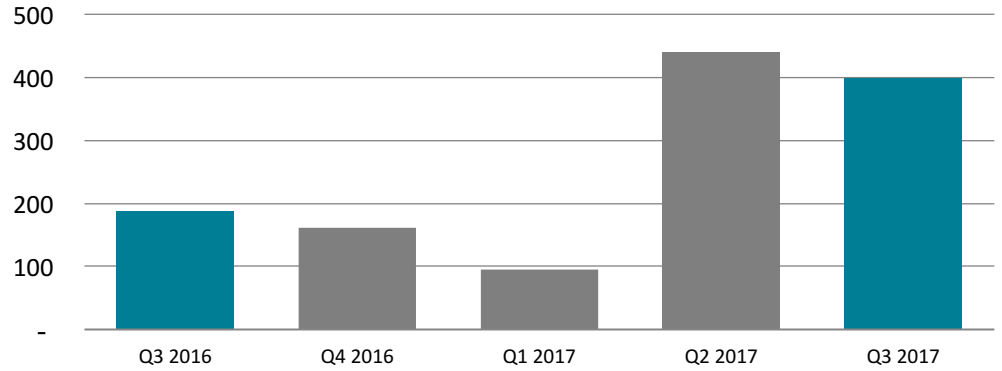
Operating loss



Cash flow



Cash position



- OPEX sequentially increased by 8% in Q317 over Q217 as recruitment to our clinical studies is ramping up
- Robust cash position gives runway to deliver key clinical read outs on our ongoing clinical studies.

Source: BerGenBio ASA Third Quarter Results

Summary and outlook / Investment case

- First-in-class AXL inhibitors for aggressive cancers with addressable market in excess of $UPDATE
- BGB324 in multiple Phase II programmes with interim data readout @ mid 2018
- Safety and proof of concept as single agent and in combination with docetaxel and erlotinib
- Well resourced & experienced organisation to deliver milestones
- Clear strategy to develop and commercialise assets

Thank you.

For further information please visit
www.bergenbio.com

Developing first-in-class Axl inhibitors to treat aggressive cancer

BerGenBio