Stemline Therapeutics, Inc.

NASDAQ: STML

Corporate Presentation

March 2016

Forward-Looking Statements

This presentation includes statements that are, or may be deemed, ‘‘forward-looking statements.’’ In some cases, these forward-looking statements can be identified by the use of forward-looking terminology, including the terms “believes,” “potentially,” “estimates,” “anticipates,” “expects,” “plans,” “intends,” “may,” “could,” “might,” “will,” “should,” “approximately” or, in each case, their negative or other variations thereon or comparable terminology, although not all forward-looking statements contain these words. They appear in a number of places throughout this presentation and include statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations.

You should read carefully our “Special Cautionary Notice Regarding Forward-Looking Statements” and the factors described in the “Risk Factors” sections of our reports on Form 10-K and Form 10-Q filed with the Securities and Exchange Commission to better understand the risks and uncertainties inherent in our business.

Mission

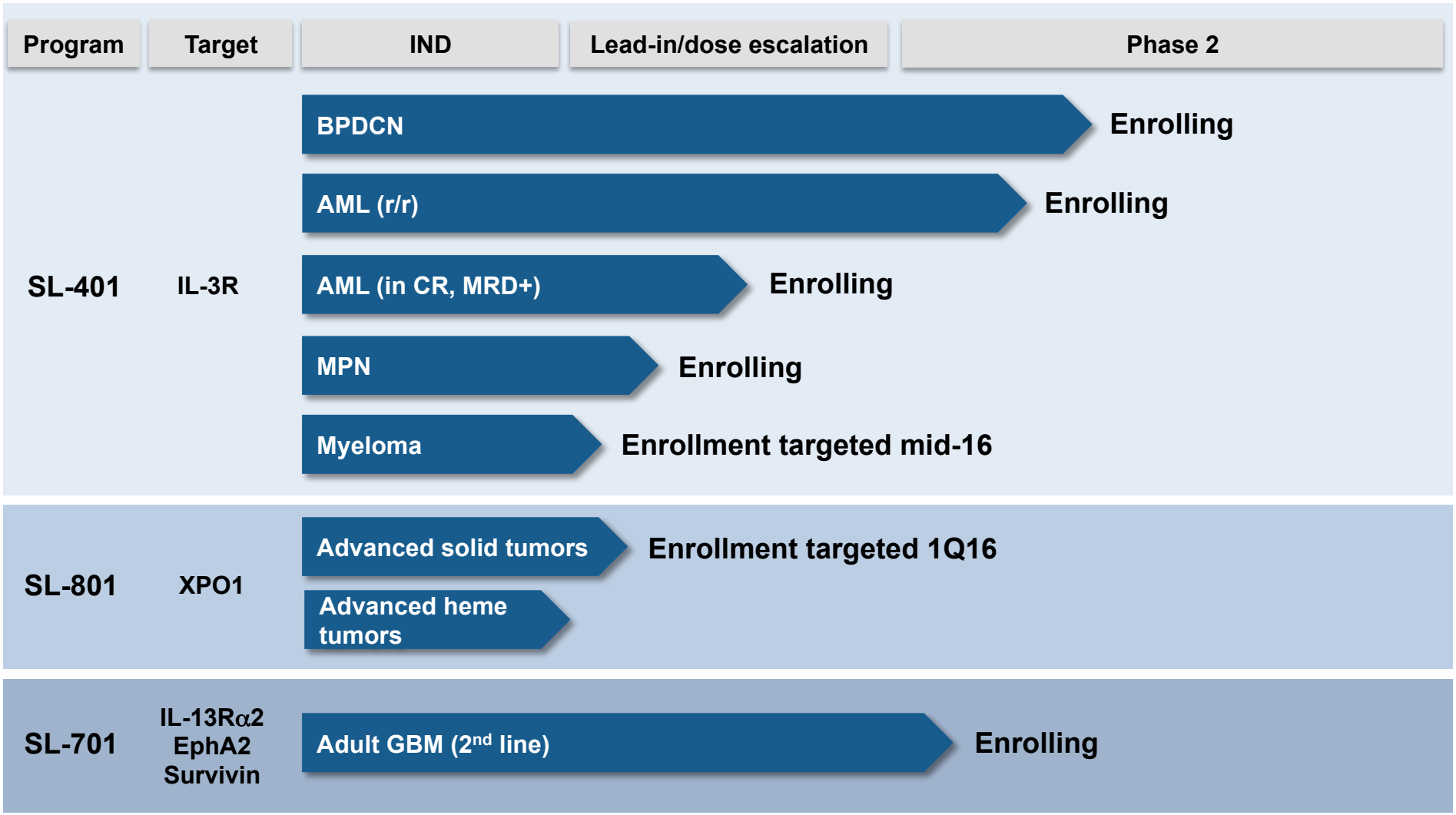
To build a leading biopharmaceutical company focused on greatly improving the lives of cancer patients by developing and commercializing innovative oncology therapeutics.

Corporate Overview

- **SL-401 potentially pivotal Phase 2 trial in BPDCN**
  - Strong enrollment (n=20); target 40-45 patients
  - Robust single agent activity
    - 87% (13/15) ORR in all-lines; 100% (10/10) ORR in first-line
    - Multiple complete responses (CR)
    - Response duration data maturing and encouraging
  - Endpoints to support potential approval: ORR, CR, PFS, OS
  - Registrational opportunities in first-line & relapsed/refractory (r/r) BPDCN
- **SL-401 market expansion opportunities**
  - Acute myeloid leukemia (AML) in CR with minimal residual disease (MRD)
  - High risk myeloproliferative neoplasms (MPN)
- **SL-701 - Immunotherapy**
  - Phase 2 in adult second-line glioblastoma (GBM)
- **SL-801 - XPO1 inhibitor**
  - Phase 1 in advanced solid tumors – enrollment targeted to begin 1Q16
- **Sufficient cash to fund through key clinical and regulatory milestones**

BPDCN=blastic plasmacytoid dendritic cell neoplasm; ORR=overall response rate; PFS=progression free survival; OS=overall survival

Pipeline



BPDCN=blastic plasmacytoid dendritic cell neoplasm; AML=acute myeloid leukemia; r/r=relapsed/refractory; CR=complete response; MRD=minimal residual disease; MPN=myeloproliferative neoplasms; GBM=glioblastoma multiforme

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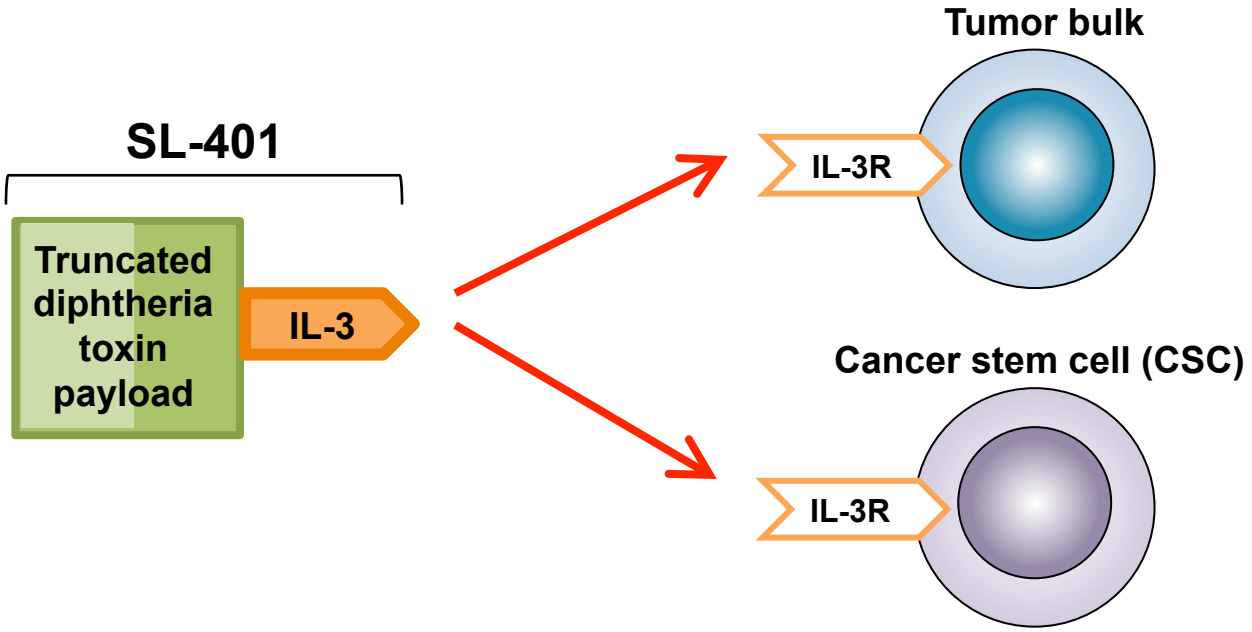
SL-401

SL-401 Targeted Therapy



IL-3R (CD123) is expressed on many hematologic cancers

- Leukemias
  - AML, MDS, CML, ALL, et al
- Lymphomas
  - Hodgkin's lymphoma, certain Non-Hodgkin's lymphoma (NHL)
- Additional hematologic malignancies
  - BPDCN
  - Myeloproliferative neoplasms (MPN)
  - Myeloma

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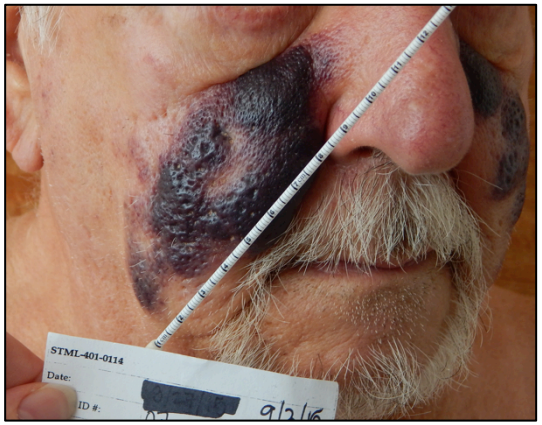
BPDCN Disease and Rationale for SL-401

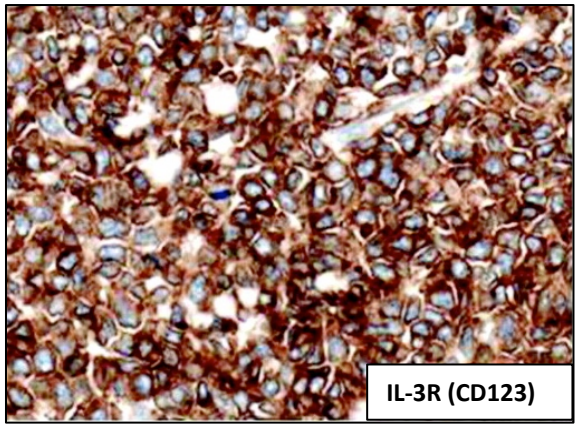
BPDCN is a highly aggressive malignancy of unmet medical need

- Multi-organ involvement: skin, bone marrow, lymph nodes, spleen, other
- Very poor prognosis with no accepted standard of care

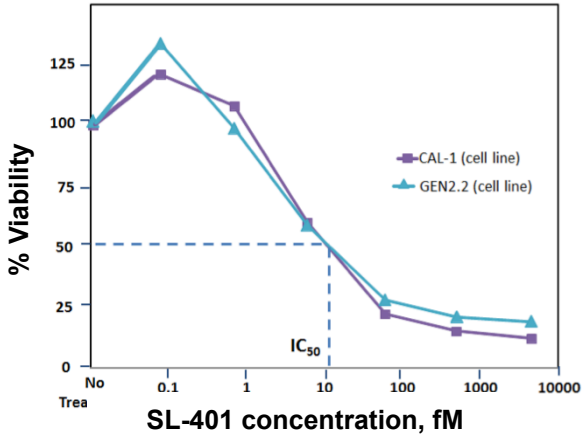
BPDCN skin lesions



Elevated IL-3R is expression
(IHC of BPDCN skin lesion)



SL-401 highly potent against BPDCN (femtomolar IC_{50})



SL-401 demonstrated robust single agent activity in previous Phase 1/2 trial

- Single cycle SL-401 had major responses, including CRs, in BPDCN and AML
  - Published in *Blood* 124: 385–392, 2014
- ***2 BPDCN pts remission >2 yrs***

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SL-401 Trial in BPDCN

- **Trial design: Phase 2 registration-directed study**

Lead-in (stage 1) completed

- BPDCN (n=9); r/r AML (n=14)
- SL-401 daily IV infusion
- At 7, 9, 12, or 16 ug/kg/day for up to 5 doses
- Repeated every 21 days

Expansion (stage 2) ongoing

- BPDCN (n=11; ongoing)
- SL-401 daily IV infusion
- At recommended stage 1 dose (12 ug/kg/day) for up to 5 doses
- Repeated every 21 days

- **Robust single agent activity**
  - 87% (13/15) ORR
    - 100% (10/10) ORR in first-line BPDCN; 8 CR and 1 CRc
    - 60% (3/5) ORR in r/r BPDCN
  - 100% (8/8) CR/CRc rate in first-line treated at 12 ug/kg/day (7 CR, 1 CRc)
    - 4 pts in remission on SL-401 and 2 pts bridged to stem cell transplant (SCT)
    - Response duration data maturing, encouraging
- **Safety profile acceptable**
  - Side effects largely transient, includes transaminitis, thrombocytopenia
  - Capillary leak managed with pre-emptive measures
  - No evidence of cumulative side effects with multiple cycles
- **Next Steps**
  - Target enrollment 40-45 patients (20 enrolled to date)
  - Data and regulatory updates throughout the year

CRc (clinical CR) = No detectable disease in bone marrow, lymph nodes or viscera with microscopic-only skin disease

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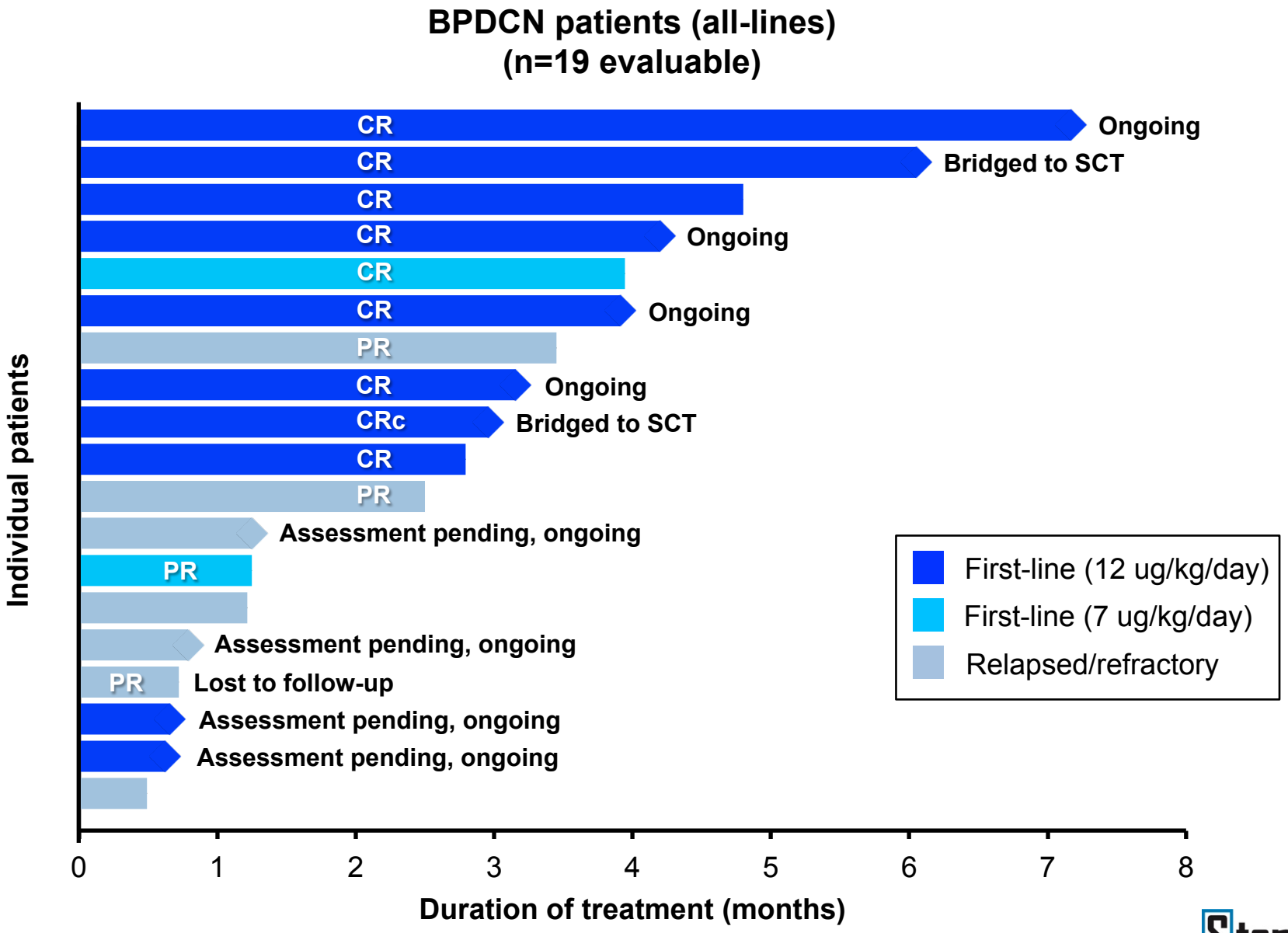
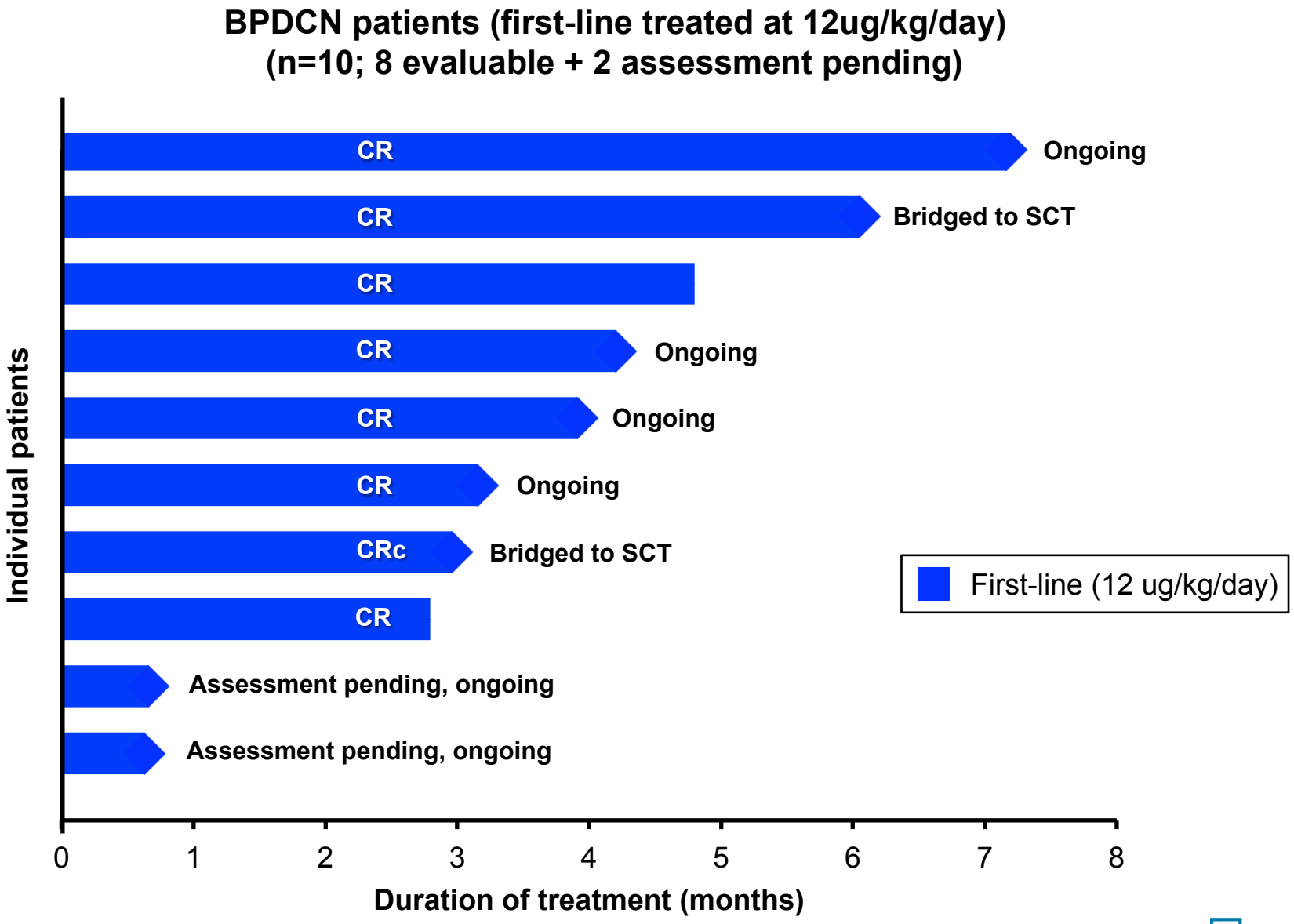
Duration of Treatment and Responses



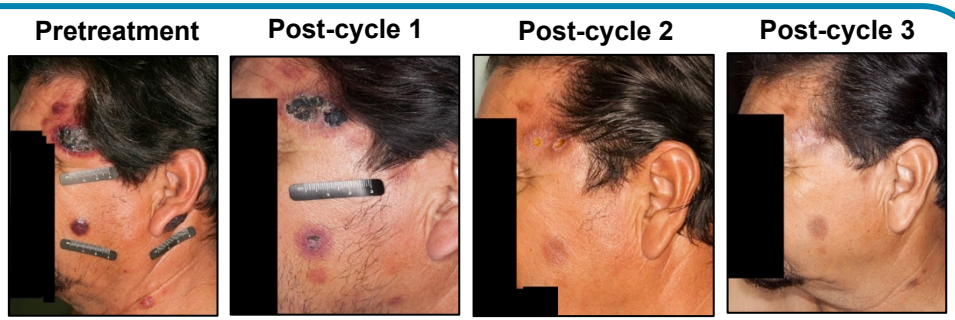
Duration of Treatment and Responses



Skin and Visceral Responses

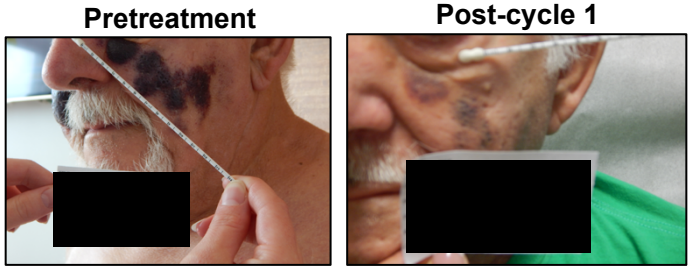
Representative skin response #1

- 63 year old male with extensive BPDCN involving skin, bone marrow and lymph nodes
- Received 6 cycles of SL-401 and achieved a CRc which included a CR in the bone marrow and lymph nodes, with resolution of gross skin lesions and positive residual microscopic skin biopsy



Representative skin response #2

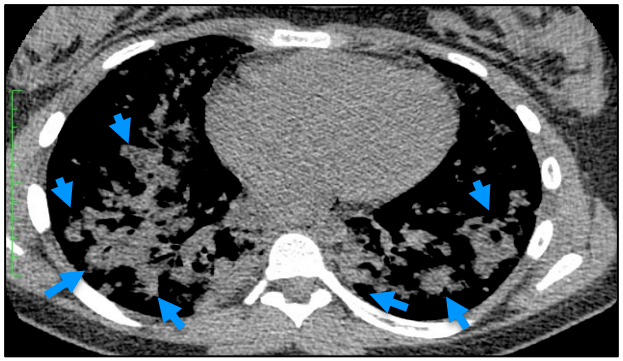
- 75 year old male with r/r BPDCN involving skin
- Received 1 cycle of SL-401 and achieved a PR with >75% reduction of gross skin lesions by mSWAT analysis



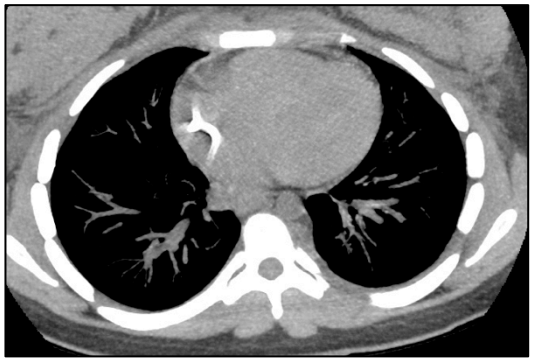
Representative visceral (organ) response: lung involvement

- 15 year old female with r/r BPDCN involving skin and bone marrow and requiring supplemental oxygen for extensive pulmonary involvement
- Received 2 cycles of SL-401 and achieved a PR with improvement of pulmonary lesions

Pretreatment
(bulky disease indicated with blue arrows)



Post-cycle 2
(normal-appearing bronchial structures)



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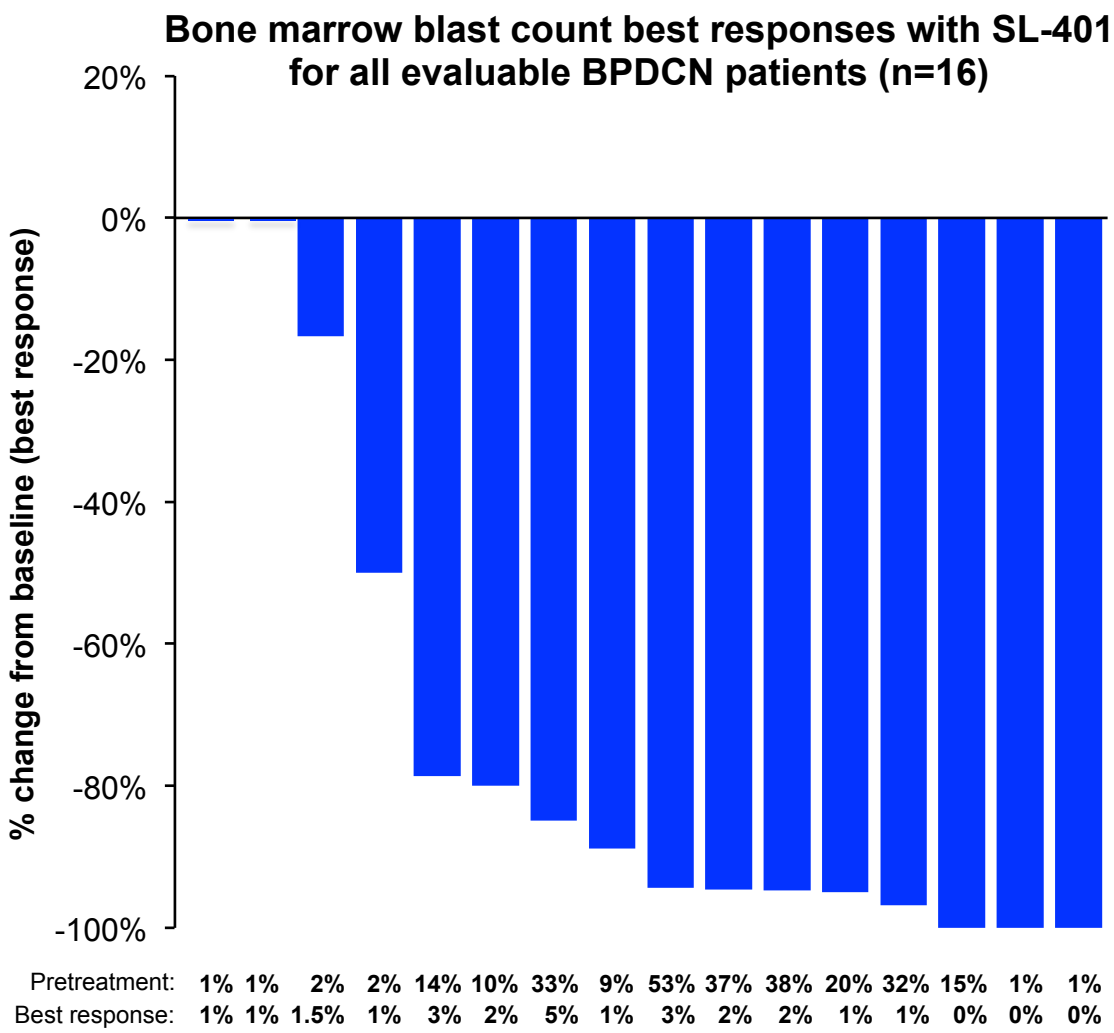
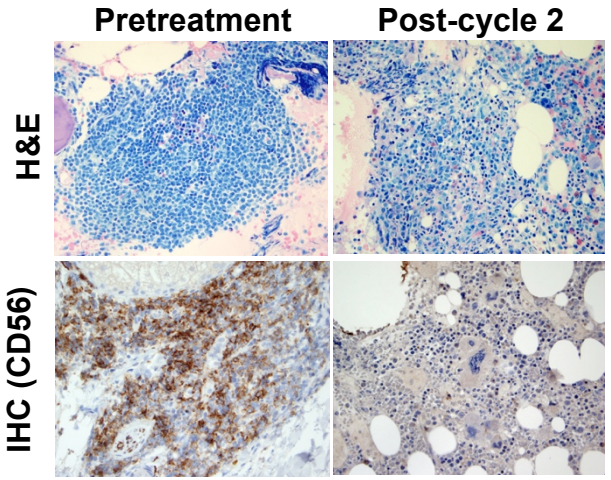
Bone Marrow Responses



Representative bone marrow response



- 62 year old female with extensive BPDCN involving skin, bone marrow, lymph nodes, viscera (spleen, eyelids, gums)
- Received 4 cycles of SL-401 and achieved a CR
- Bone marrow biopsy (pretreatment and end of cycle 2) shows clearance of CD56+ BPDCN cells

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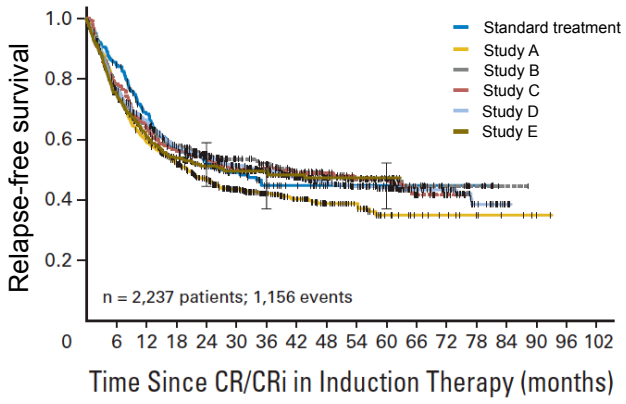
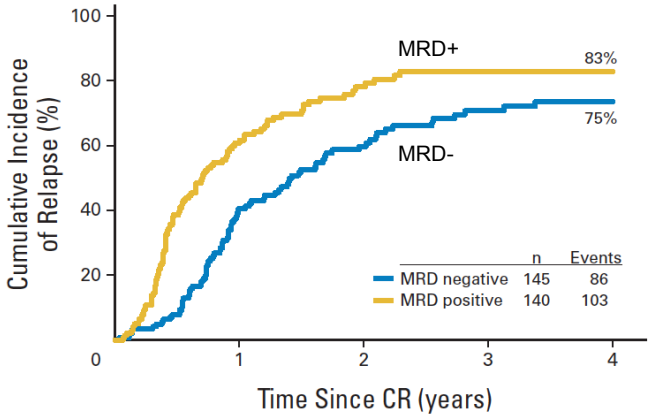
Rationale for SL-401 in AML in CR, MRD+

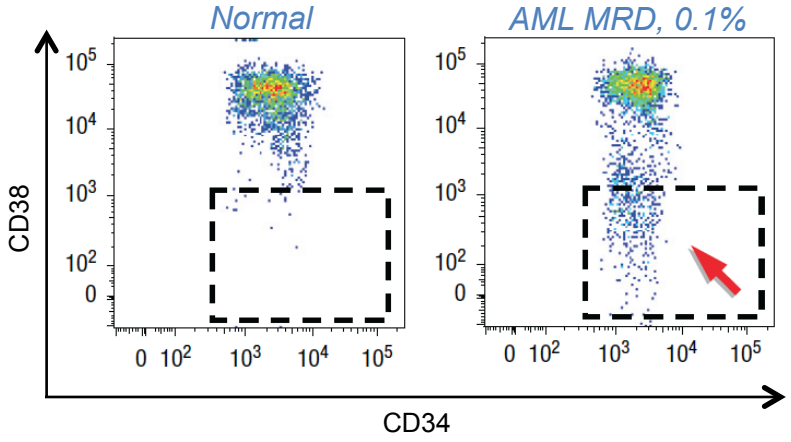
Majority of AML patients in 1st CR will relapse



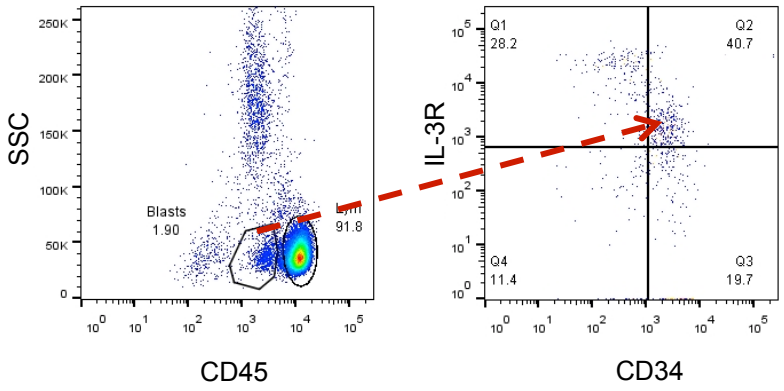
MRD is a predictor of 1st relapse



MRD is CSC-rich



MRD is IL-3R+



Buchner, T. JCO, 2012; Freeman, S. D. JCO, 2013; Jorgensen, J. L. Clin Lymphoma Myeloma Leuk, 2011; Konopleva, M. (unpublished)

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AML in CR, MRD+ Trial

Stage 1 (Lead-in) - open

- AML in CR
- ~9-12 patients
- 7, 9, or 12 ug/kg/day for 5 days, every 4 weeks
- ~15 sites in North America

Stage 2 (Expansion)

- AML in CR, MRD+
- ~15-20 patients
- At dose defined by Stage 1
- Endpoints
  - MRD+ to MRD- conversion
  - CR durability

Updates expected mid-16

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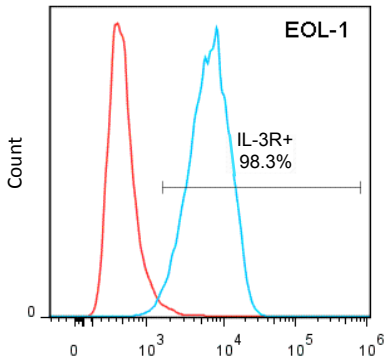
Rationale for SL-401 in MPN (Mastocytosis, Eosinophilic syndrome, Myelofibrosis, CMML)

IL-3R expression on mastocytosis

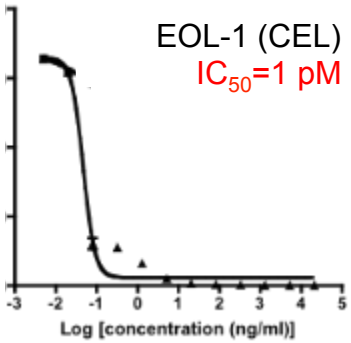
Systemic mastocytosis	IL-3R (CD123+)	pDC proliferation in microenviroment
All types	64% (37/58)	76% (44/58)
• Aggressive (ASM)	• 100% (10/10)	• 90% (9/10)
• Indolent (ISM)	• 61% (14/23)	• 87% (20/23)
• With associated heme malignancy (SM-AHN)	• 57% (13/23)	• 65% (15/23)
• Mast cell leukemia (MCL)	• 0% (0/2)	• 0% (0/2)

IL-3R expression on eosinophilic leukemia and potent SL-401 anti-tumor activity

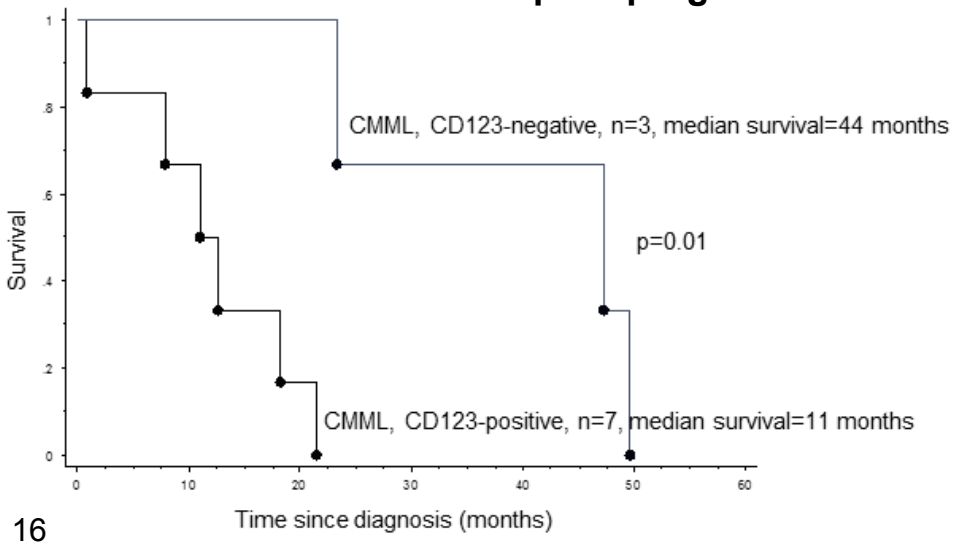
IL-3R expression



SL-401 activity



IL-3R (CD123) expression by CMML correlates with poor prognosis



Pardanani. Leukemia, '15; ASH, 2015

MPN Trial

Stage 1 (Lead-in) - open

- 4 types of high-risk MPN*
- ~9-12 patients
- 7, 9, or 12 ug/kg/day for 3 days, every 3 weeks
- 10-15 sites in North America

Stage 2 (Expansion)

- 4 types of high-risk MPN
  - 4 arms (1 arm for each indication
- ~15-20 patients each arm, with expansion flexibility (Simon 2-stage)
- At dose defined by Stage 1
- Endpoints: ORR, CR, response duration

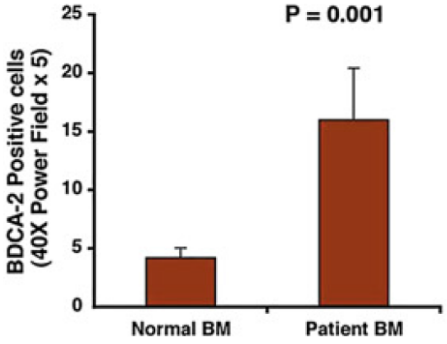
Updates expected 2H16

<u>*4 types of high-risk myeloproliferative neoplasms (MPN)</u>

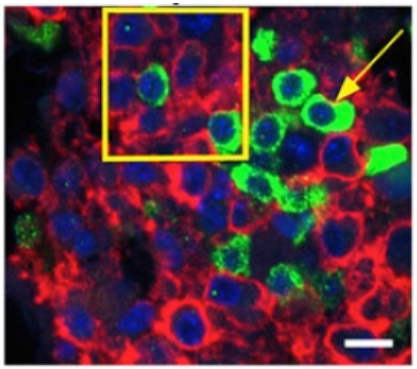
- Mastocytosis
- Eosinophilic syndrome
- Myelofibrosis
- Chronic myelomonocytic leukemia (CMML)

Rationale for SL-401 in Myeloma

pDCs are elevated in myeloma bone marrow

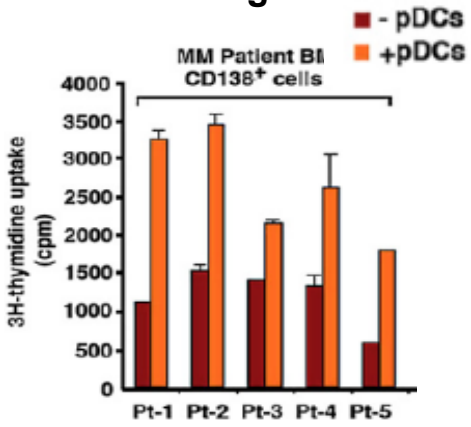


pDCs and MM cells direct contact in vivo

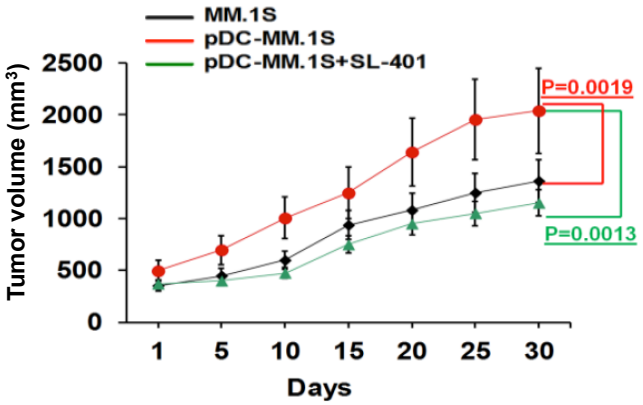


MM patient BM biopsy

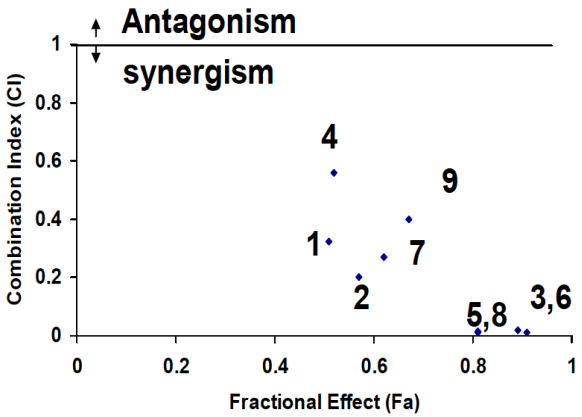
pDCs potentiate MM cell growth



SL-401 inhibits pDC-induced MM cell growth in vivo



SL-401 is synergistic with pomalidomide



ASCO, 2014; Chauhan. Cancer Cell, 2009
Collaboration with Dana-Farber

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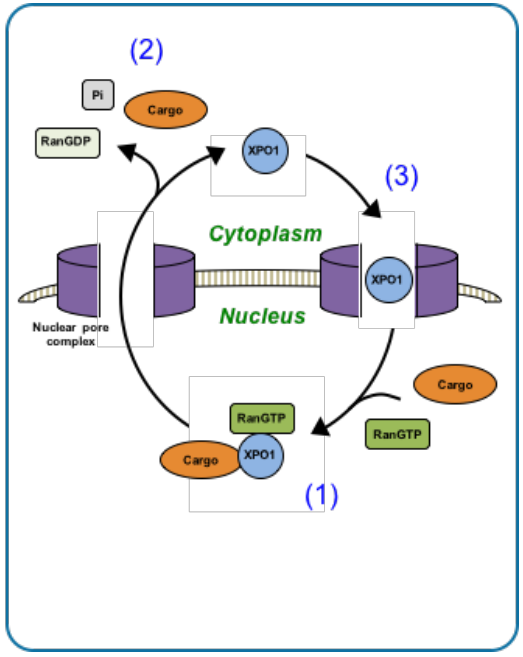
SL-801

SL-801 - Novel XPO1 Inhibitor

On track to treat first patient this quarter



1. XPO1 recognizes cargo proteins through nuclear export sequences and bind cargos in nucleus.
2. Ternary complex transported through nuclear pore complex and into cytoplasm where cargo released.
3. XPO1 and Ran subsequently recycled into nucleus where process is repeated.

SL-801 – Oral Reversible XPO1 Inhibitor

- Oral, reversible small molecule inhibitor of Exportin 1 (XPO1)
- XPO1 is a key transport protein involved in nuclear export of tumor suppressor and oncogenic proteins
- XPO1 overexpression associated with aggressive characteristics and poor outcome in many solid and hematologic cancers

SL-801 Has Clinically Validated Mechanism of Action

- Reversible XPO1 inhibitor
- IC50 in low nanomolar range
- Induces XPO1 degradation
- Attributes may offer improved safety and dosing flexibility

Status – IND Active; First Patient Treated this Quarter

- Evaluating in advanced solid tumors then hematologic cancers

Strong IP protection

- Composition of matter patent to 2030

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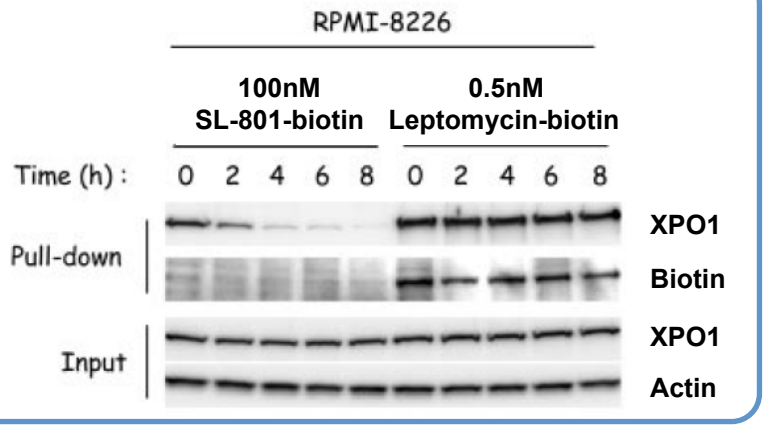
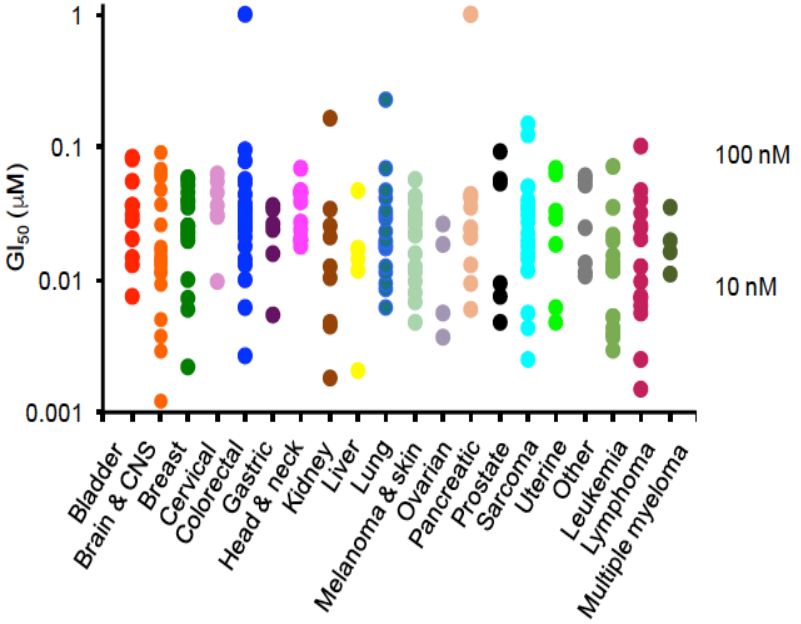
SL-801 - Reversible and Potent XPO1 Inhibitor

SL-801 is a reversible inhibitor of XPO1



Source: Sakakibara, Blood 2011; ASH 2015

SL-801 has potent in vitro activity against multiple solid and hematologic cancers



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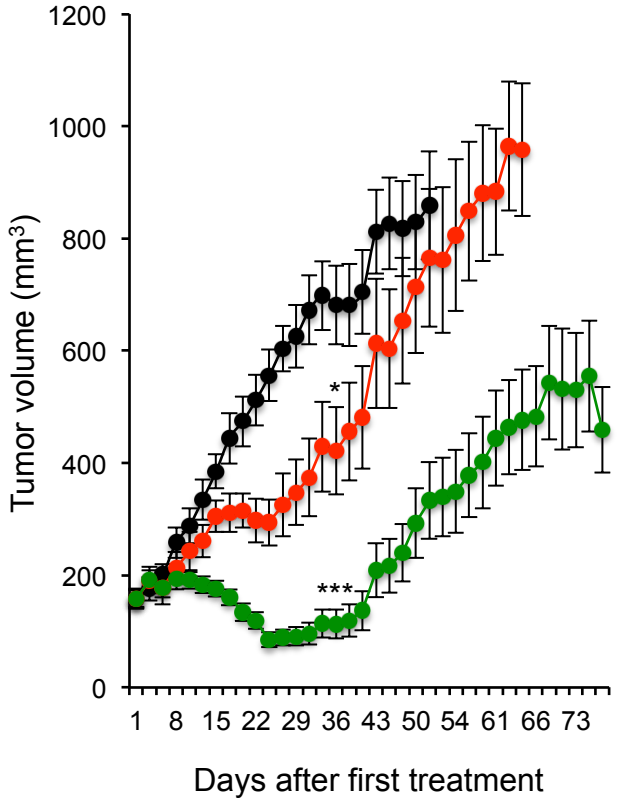
SL-801: Anti-Tumor Activity in Animals

- SL-801 demonstrates potent anti-tumor activity in animal models, across wide array of solid and hematologic cancers

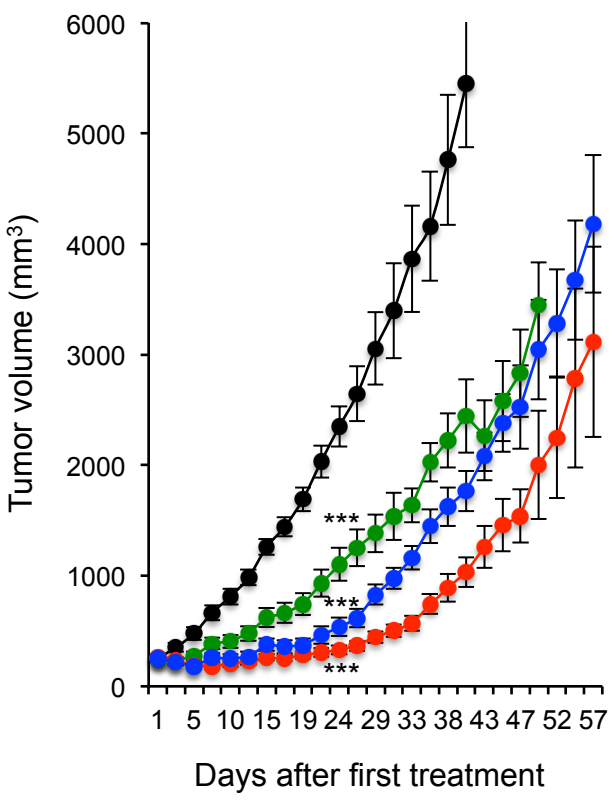
NCI-H226 non-small cell lung cancer

Vehicle, qd (d1-5,8-12,15-19)
SL-801, 31.25 mg/kg, qd (d1-5,8-12,15-19)
SL-801, 125 mg/kg, qd (d1,3,5,8,10,12,15,17,19)



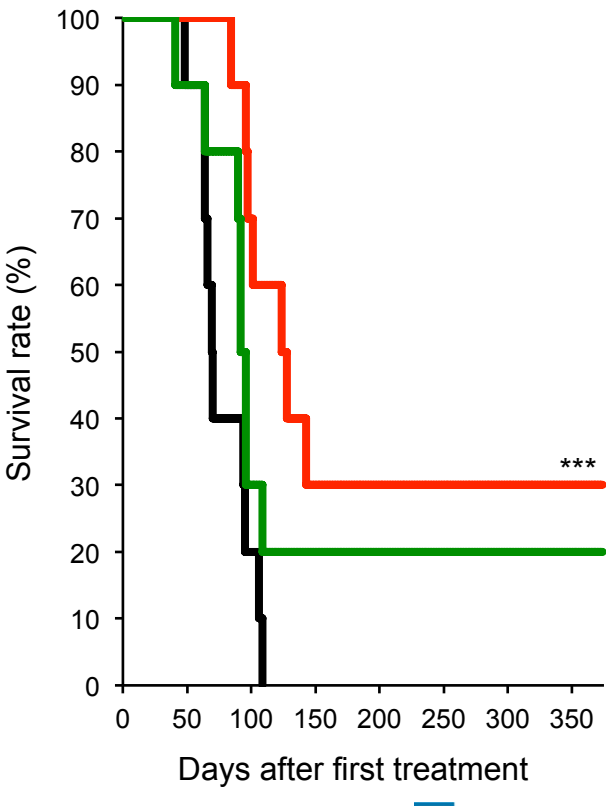
22RV prostate carcinoma

Vehicle, qd (d1,3,5,8,10,12,15,17,19)
SL-801, 125 mg/kg, qd (d1,3,5,8,10,12,15,17,19)
SL-801, 125 mg/kg, qd (d1,8,15)
SL-801, 250 mg/kg, qd (d1,8,15)



MM.1S multiple myeloma

Vehicle, qd (d1,3,5,8,10,12)
SL-801, 125 mg/kg, qd (d1,3,5,8,10,12)
SL-801, 125 mg/kg, qd (d1,8,15)



**, p < 0.05; **, p < 0.01; ***, p < 0.001*

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SL-801 – Clinical Development Plan

Phase 1 in advanced solid tumors – Enrollment targeted 1Q16

Multicenter, Dose Escalation (Standard 3+3 design)

- Advanced solid tumors
- 40-50 patients with advanced solid tumors
- Dose escalation: starting at 5mg oral tablet/day
  - 4 days on/3 days off x 2 weeks (for 21 day cycle)
- Evaluate safety
- Signal detection for subsequent Phase 2 disease-directed trials
- Endpoints
  - Safety, maximum tolerated dose (MTD) determination
  - ORR, disease control, duration of response, PFS, OS
- 5 sites in U.S.

Updates expected 2H16

➢ **Phase 1 in advanced hematologic cancers targeted 2H16**

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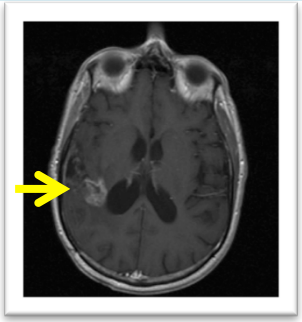
SL-701

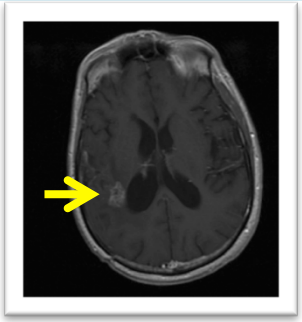
SL-701 Background

- **Immunotherapy directed to multiple tumor targets**
- **Orphan drug designation in glioma**
- **Previous investigator-sponsored Phase 1/2 trial**
  - Earlier version of SL-701 + immunostimulant adjuvant: poly-ICLC, a toll-like receptor 3 (TLR3) agonist that activates NK cells and CD8+ T cells
    - Major objective responses, including CRs, in advanced adult and pediatric brain cancer; some responses occurred late (≥12 mos of therapy)
    - Induction of immune response with SL-701 is associated with tumor regression

Pre-therapy (baseline)

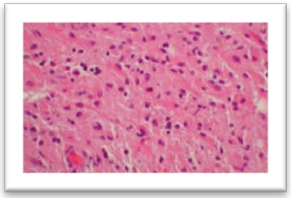


Nine weeks post-therapy shows tumor shrinkage

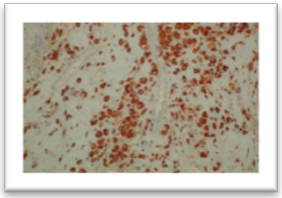


- Inflammatory response, including abundant cytotoxic ($CD8^{+)}$ T cells, in brain tissue
- Indicative of immune response against the brain tumor

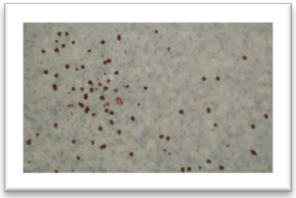
Post-therapy brain biopsy



Reactive gliosis



Numerous $CD68^{+}$ macrophages



Abundant $CD8^{+}$ T cells

Stemline

SL-701 Next Steps

- **Corporate-sponsored Phase 2 program**
  - **Initial stage:** SL-701 + different adjuvants: GM-CSF and Imiquimod
    - Patients continue to be followed for PFS and OS
  - **Next stage (enrolling):** SL-701 + poly-ICLC + bevacizumab
    - Poly-ICLC: More closely replicate previous regimen
    - Bevacizumab: Clinical support emerging that VEGF may suppress immune stimulation and thus VEGF inhibition may combine well with immunotherapeutic approaches

Stemline

Financial Summary

Financial Summary

	As of December 31, 2015
Cash, Cash Equivalents and Investments (mm)	$97.5
Debt	$0.0
Shares Outstanding (mm)	18.2

Stemline Therapeutics, Inc.

NASDAQ: STML

Corporate Presentation

March 2016