BioCryst Pharmaceuticals

Jefferies 2010 Global Life Sciences Conference – New York

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June 8, 2010

BIOCRYST PHARMACEUTICALS, INC.

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We Have the Key Elements to Build an Enduring, Successful Company

Transformed biotechnology company with product revenue and two late-stage development candidates

Hospitalized seasonal influenza is a sizable, annual revenue opportunity following peramivir regulatory approval. Any emergency use is upside.

Advancing PNP programs: positive first data in gout patients, and two fully enrolled studies–a pivotal trial for CTCL(1) and an exploratory Phase 2 for CLL(2)

Non-dilutive financing mitigates cash burn and allows investment in R&D (3)

Experienced and proven team focused on delivering

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Significant Value Creating Events to Come

Clinical Events

- Forodesine data from pivotal CTCL study and Phase 2 CLL study
- Additional data from two Phase 2 studies of BCX4208 in gout
- Data from Phase 3 studies in i.v. peramivir in hospitalized, seasonal influenza

Regulatory Events

- Potential approval of peramivir in South Korea, other countries
- Potential filing for peramivir approval in other countries

Commercial Events

- Stockpiling partners provide virtually global coverage
- Potential additional government orders for i.v. peramivir
- Potential royalties from ex-U.S. approvals

Advancing Late-Stage Pipeline in Multiple Indications

INFECTIOUS DISEASE

	PRE-CLINICAL	PHASE 1	PHASE 2	PIVOTAL	FILED	APPROVED
Peramivir Outpatient Flu (Seasonal Influenza / i.v.)	SHIONOGI & CO., LTD.					
Peramivir Inpatient Flu (Acute Influenza / i.v.)						

CANCER

	PRE-CLINICAL	PHASE 1	PHASE 2	PIVOTAL	FILED	APPROVED
Forodesine (CTCL)						
Forodesine (CLL)						
Forodesine (ALL)	mundipharma					

INFLAMMATORY DISEASE

	PRE-CLINICAL	PHASE 1	PHASE 2	PIVOTAL	FILED	APPROVED
BCX4208 (Gout)						

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PNP INHIBITORS

BCX4208 FOR GOUT

FORODESINE FOR LYMPHOMA/LEUKEMIA

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PNP Development Strategy Is Advanced and Targeted

CANCER

	PRE-CLINICAL	PHASE 1	PHASE 2	PIVOTAL	FILED	APPROVED
Forodesine (CTCL)						
Forodesine (CLL)						
Forodesine (ALL)						

GOUT

	PRE-CLINICAL	PHASE 1	PHASE 2	PIVOTAL	FILED	APPROVED
BCX4208						

Multiple pre-clinical compounds available for near-term development

Gout is a Growing Market with Significant Unmet Needs

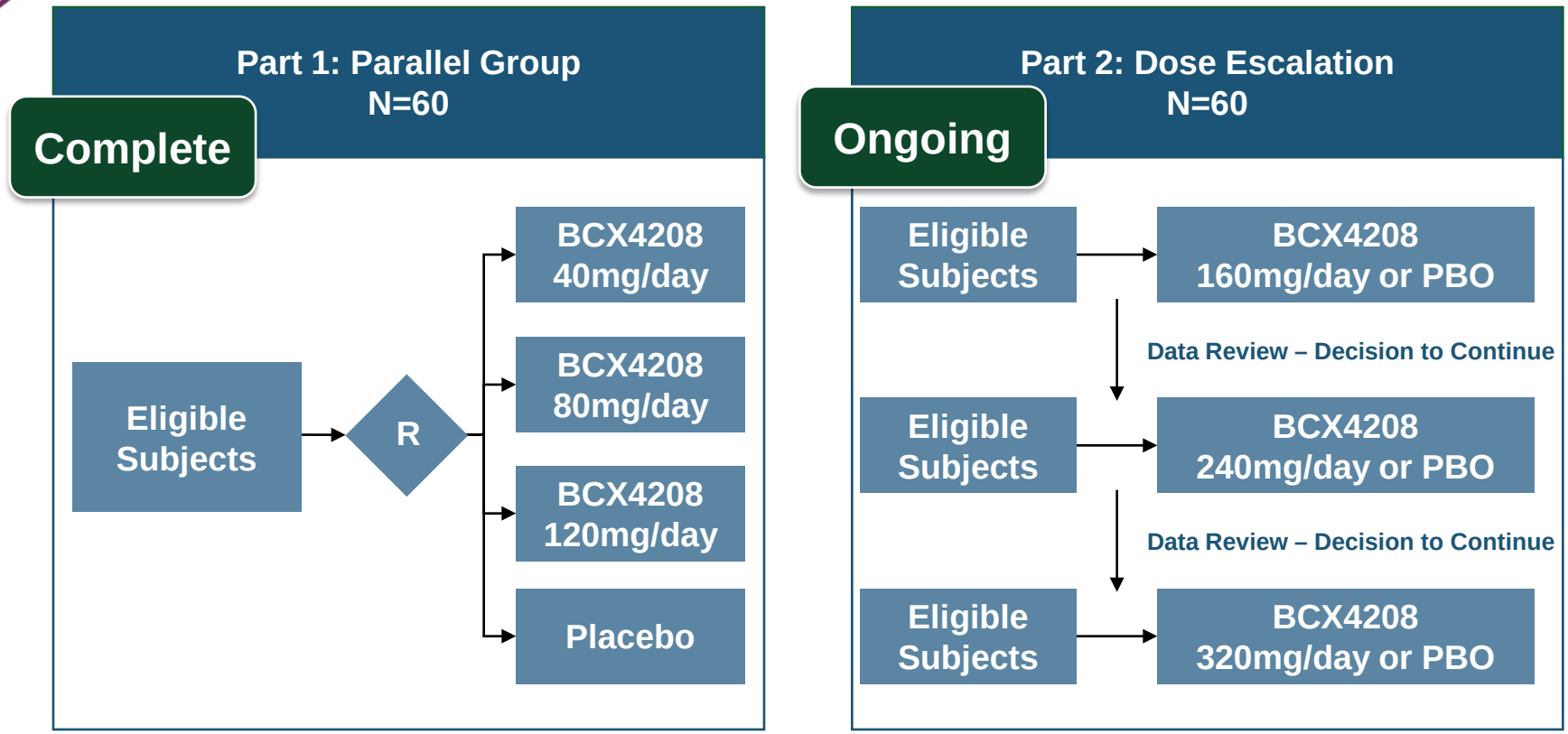
- **Gout is now the most common inflammatory arthritis in males**
- **Over last 40 years, the prevalence has increased in most Westernized countries by 200-300%**
- **Growth in prevalence of gout appears to be caused by several factors including:**
  - Alarming rise in obesity
  - Aging population
  - Increase in prevalence of kidney failure & hypertension
  - Widespread use of prescription drugs and alcohol
- **Issues with current therapies: efficacy, allergic reactions and side effects**

BCX4208 Gout Development Strategy & Commercial Rationale

Study	Design	Goal	Primary Outcome Measure	Top-line Data Expected
Monotherapy Dose-ranging Phase 2a	Randomized, double-blind, placebo-controlled	Dose-response + Safety	Reduction in uric acid	Top-line data reported 1Q:10
Combination therapy Phase 2b	Randomized, double-blind, multi-center, placebo-controlled (BCX4208 +/- Allopurinol)	Dose-response + Safety	Reduction in uric acid	4Q:10

- Gout prevalence in the U.S. and Europe ranges from 0.8-1.4%
- U.S. demographic lifestyle trends could lead to 3.8-5.2M with gout in 2020
- ~$2000 annually for current gout therapy (*febuxostat*)
- New products for refractory gout may price as high as $20,000/yr

Phase 2a Gout Study – Dosing Design and Research Goals



Study Goals

- Determine dose response for uric acid reduction (efficacy)
- Determine dose response for reduction in lymphocytes (safety)
- Select a dose(s) for Phase 2b trial

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BCX4208 Phase 2a, Part 1 in Gout Patients: Efficacy Results

Once-Daily Treatment:	Placebo N=15	BCX4208		
		40mg N=15	80mg N=14	120mg N=16
Primary Endpoint: Change in Uric Acid from Baseline at day 22				
All subjects	-0.4mg/dL (-4.2%)	-2.7mg/dL (-32.2%)	-3.3mg/dL (-34.6%)	-3.4mg/dL (-33.7%)
P value vs placebo		p<0.001	p<0.001	p<0.001
Proportion of Subjects With Uric Acid Levels < 6mg/dL at day 22				
All subjects	0%	33%	36%	31%
P value vs placebo		p<0.05	p<0.05	p<0.05
Subjects with baseline serum UA <10 mg/dL	0% N = 10	38% N = 13	30% N = 10	63% N = 8

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BCX4208 Phase 2a, Part 1 in Gout Patients: Safety Results

- Overall, the frequency of adverse events in each of the BCX4208 treatment groups was comparable to that observed in the placebo group
- All 60 subjects enrolled completed the first part of this study
- The protocol included stopping rules for CD4+ cell counts below certain thresholds
  - No subjects were discontinued for this reason
  - Additional studies designed to evaluate longer-term exposure are needed to further define the safety and tolerability profile of BCX4208
- The incidence of gout flares observed was low
  - All patients received prophylactic medicine for gout flares

BCX4208 was generally safe and well-tolerated over the range of 40 mg to 120 mg daily for three weeks

Phase 2 Combination Study of BCX4208 in Gout Patients

Study Design

- Utilizes a classical Latin square factorial design
- Randomized, double-blind, multi-center, placebo-controlled study
- Evaluates BCX4208 at doses of 20 mg, 40 mg and 80 mg alone or in combination with allopurinol at doses of 100 mg, 200 mg and 300 mg, administered once-daily for 21 days
- Powered at 80% for the primary endpoint
- Enrollment target is ~80 patients

Study Goals

- Evaluate the urate-lowering activity and safety of several doses of BCX4208 alone and in combination with selected doses of allopurinol
- Efficacy will be assessed during the study by means of serum uric acid concentrations
- Design allows detection of synergistic or additive effects of combination therapy

Gout Phase 2 combo study is currently enrolling, data expected 4Q:10

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Existing CTCL Treatments Have Significant Limitations

- Many patients with CTCL have a prolonged course of illness and quality of life is an important consideration
  - Treatment should be effective, safe, convenient and tolerable for long-term chronic use
- Majority of current treatment options have unfavorable side effect profiles and are inconvenient
- Approved oral agents have dosing challenges and difficult side effect profiles

Forodesine profile meets patient needs in the CTCL market

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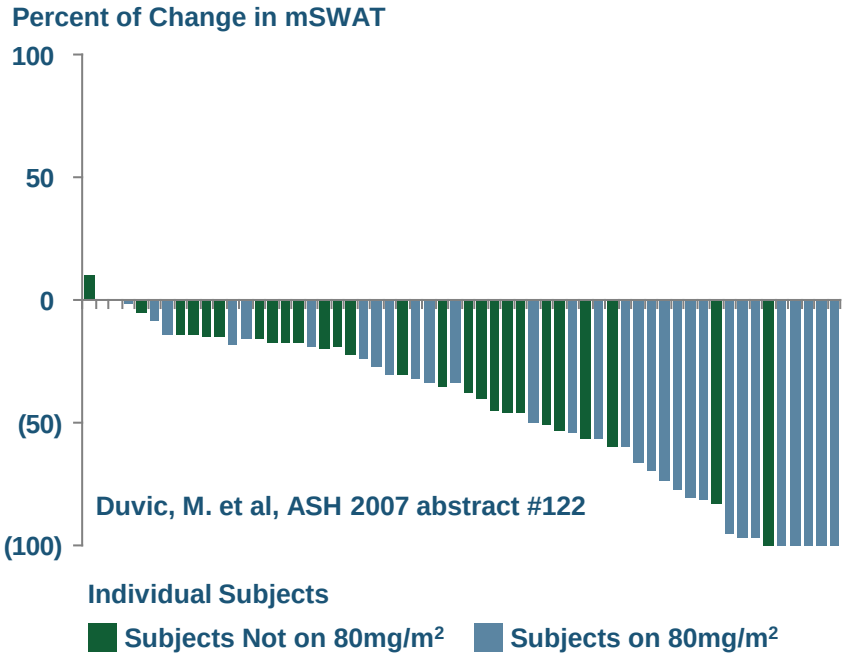
Proof of Concept for Forodesine in CTCL

Best mSWAT from Baseline in All Subjects vs. 80mg/m^2 (Blue)



Data from BCX1777-105 study

Outcome Measure	80 mg/m^2 N=36
Overall RR	14 (39%)
CR	2 (6%)
PR	12 (33%)
SD	15 (42%)
Time to Response, Days (95% CI)	42 (29.0 – 58.0)
Response Duration, Days (95% CI)	127 (71.0, N/A)

Long-term treatment data presented at ASCO 2009 demonstrated acceptable safety profile and efficacy in CTCL subjects treated for 12+ months

Solid Phase 1 – 2 results support pivotal study

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Forodesine CTCL Pivotal Trial Nearing Completion

Pivotal study is ongoing under special protocol assessment by FDA

Study Design

- Single arm
- Stages 1b through 4
- Three or more failed treatments
- Prior therapy must include bexarotene

Intervention & Outcome Measure

- Forodesine 200mg daily
- Primary outcome measure is objective response rate

Commercial Rationale

- Differentiated profile
- Market addressable by small sales force
- Composition of matter IP through 2017
- Potential price premium
- ~$80M sales of approved CTCL products over the last 12 months

Pivotal CTCL study fully enrolled, data expected 2H:10

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CLL is an Attractive Indication for Forodesine

- Most of the currently available drugs have significant toxicity concerns and i.v. dosing

- Forodesine for CLL
  - Novel mechanism of action
  - Generally safe and well-tolerated in clinical trials
  - Orally administered
  - Suitable for combination and potentially synergistic with existing therapies

People living with CLL in U.S., 2008: 90,179

New CLL diagnoses in U.S., 2008: 15,110

$35K-$75K /patient spent on CLL therapy annually

Incidence data: American Cancer Society

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PERAMIVIR FOR INFLUENZA

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Peramivir (RAPIACTA®) Approved & Launching in Japan

- **First marketing approval of a BioCryst discovered drug – January 2010**
- **Broad Indication**
  - Treatment of viral infection with influenza type A and type B
  - Seasonal, for patients with uncomplicated flu (single administration)
  - Seasonal, for patients with a risk of increased severity due to complications (multiple dose administration)
- **One of fastest review periods ever by Japanese authorities**
- **Japan is one of world's largest markets for influenza anti-virals**
  - Over 10 million people treated annually
  - Gov't panel recommendation: grow anti-viral stockpile to cover 60 million citizens
- **Attractive economics for BioCryst**

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New Influenza Anti-Virals are Needed

- **Seasonal influenza requiring hospitalization is a serious medical problem**
  - 200,000 hospitalizations
  - 36,000 deaths in the U.S. annually, according to CDC
- **No anti-viral is approved for hospitalized influenza patients**
- **Currently approved anti-virals are dosed orally or inhaled**
- **Intravenous delivery of therapy is often the preferred route to treat infections in the hospital setting**

This medical need can be addressed by a potent, rapidly delivered, safe, effective i.v. anti-viral

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Key Requirements for U.S. NDA Filing

Task	Status
Two adequate & well-controlled efficacy studies	One completed One in progress
1,500 subjects for safety data base	> 1,000 already complete
Commercial scale CMC	NDA lots completed
Drug product shelf life	Currently > 3 years

Placebo-controlled Shionogi Phase 2 study is 1 of 2 well-controlled studies needed for approval

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Peramivir Phase 3 Program Enrolling

Parameter	Study 301	Study 303
Dose Groups	PVR 600mg QD x 5 days + Std of Care *vs.* Placebo + Std of Care	PVR 300mg BID x 5 days: or PVR 600mg QD x 5 days
Allocation ratio	2:1	1:1
Power	90%	N/A
Hazard ratio	0.67	N/A
Endpoint	Time to Clinical Resolution	Reduction in viral titer
N Total	Sufficient to confirm 306 in ITTI (est. 445)	300
Patient Population	Requires hospitalization and presence of one or more risk factors	Broad inclusion
Seasons	NH / SH ’09/’10, NH ’10/’11	NH / SH ’09/’10, NH ’10/’11

Additional studies to provide further evidence of efficacy are under discussion with FDA & HHS

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Sustainable Revenue Opportunity—Seasonal Flu in the Hospital

- **Global flu season:**
  - 3-5 million severe illnesses
  - 250-500,000 deaths
- **Potentially the first i.v. anti-viral to market**
- **Pharmacoeconomic benefits in reducing hospital and ICU stays**
- **Patent protection to 2018**
- **Revenue generation for BioCryst from Shionogi**
  - $7 million milestone for approval in 1Q 2010
  - Royalties on net sales of 10-20%
  - Potential future commercial milestones of up to $95 million

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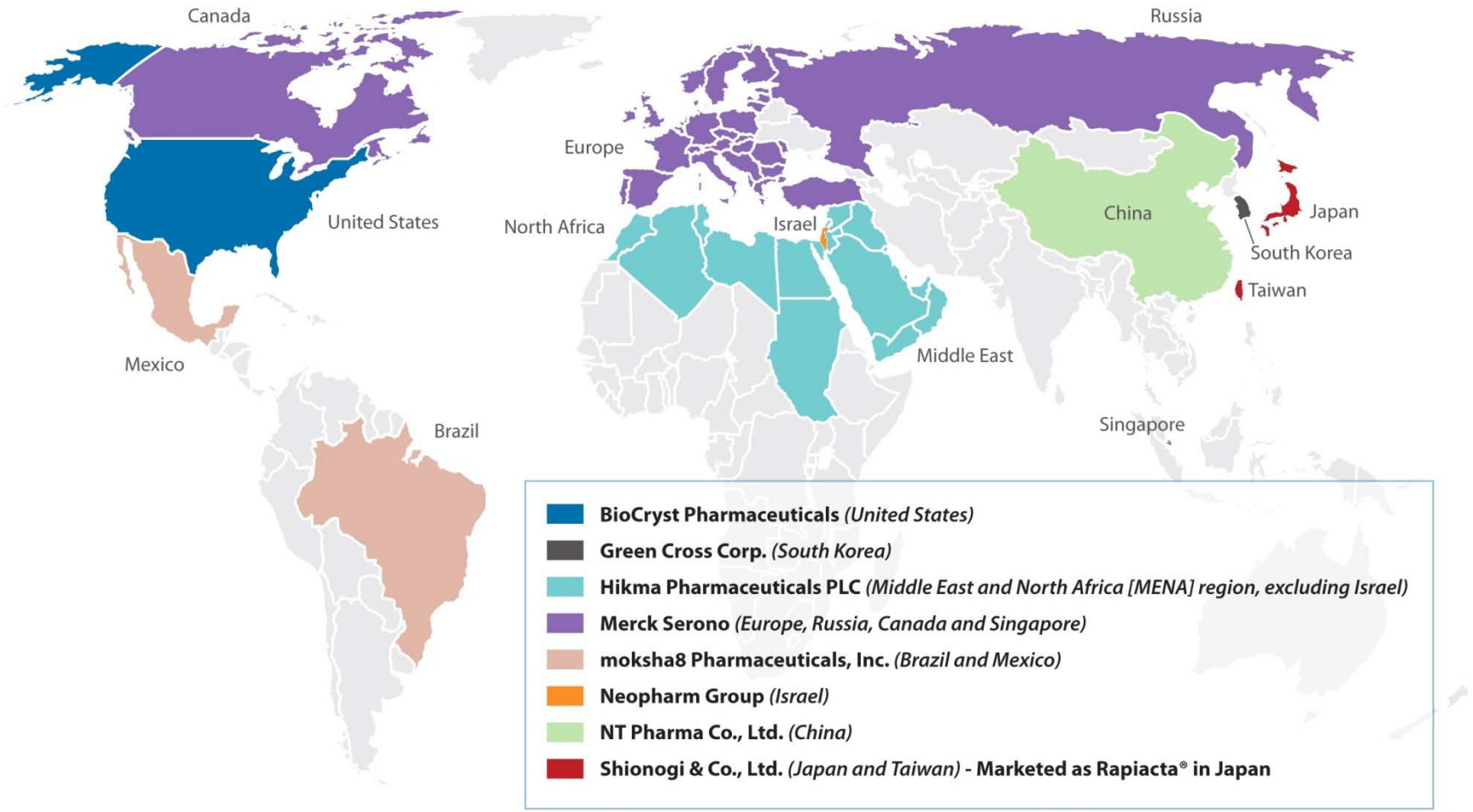
Stockpiling Partners Provide Virtually Global Coverage



Near-Term Peramivir Revenue Opportunities

Full Regulatory Approval

Recurring royalties from Shionogi sales

Filed for approval in S. Korea, pediatric label expansion in Japan

Other ex-U.S. potential filings/approvals

Government Stockpiling

Potential additional orders from HHS

Potential ex-U.S. Government orders through BioCryst and regional partners

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Selectively Investing in our Discovery Platform

Target	Illustrative Indications
Hepatitis C RNA Polymerase	Chronic Hepatitis C
Kallikrein	Hereditary angioneurotic edema
Complement	Age related macular degeneration
JAK	Psoriasis; Rheumatoid arthritis

- Management has focused on advancing late-stage products
- Successful clinical progress and non-dilutive funding allow the opportunity to invest in early stage assets
- Multiple promising candidates in development
- Management is taking a focused and financially disciplined approach to R&D

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Financial Summary

- Potential revenue opportunities exist from:
  - Royalty payments from strategic partnerships
  - Revenue from peramivir stockpiling
- Recent secondary offering & product revenue have significantly strengthened BioCryst's balance sheet
- Current cash allows us to fund clinical development plans for the next 2-3 years

Common shares outstanding	**44.0 M as of 3/31/10**
Cash, cash equivalents & securities	**$89.4 M as of 3/31/10**
Underlying 2009 cash use*	**$37.2 M**
Anticipated 2010 cash use	**$25-30 M**

**Excludes proceeds from equity offering and sale of peramivir to HHS*

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2010 Events

Clinical Events

- ✓ Additional stockpiling partners covers major markets globally
- ✓ Update regarding forodesine CLL study
- ✓ Forodesine CTCL study reaches full enrollment (1Q:10)
- ✓ Data from Phase 2 monotherapy study of BCX4208 in gout (2Q:10)
- ✓ Provide update: plans for BCX4208/allopurinol combo study in gout (2Q:10)
- ☐ Data from pivotal forodesine CTCL study (2H:10)
- ☐ Data from Phase 2 BCX4208/allopurinol combo study in gout (4Q:10)
- ☐ Data from Phase 2 CLL study (2H:10)

Regulatory and Commercial Events

- ✓ Peramivir marketing authorization in Japan
- ☐ Potential for additional regulatory approvals and revenue events
- ☐ Potential for royalty payment on seasonal sales of peramivir ex-U.S.

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