Arno Therapeutics

Developing a Novel Targeted Cancer Treatment

Corporate Presentation
June 2014
This presentation contains forward-looking statements that involve substantial risks and uncertainties. These statements are often, but not always, made through the use of words or phrases such as "anticipates," "expects," "plans," "believes," "intends," and similar words or phrases. All statements, other than statements of historical facts, included in this presentation regarding our strategy, future operations, outlook, milestones, the success of Arno’s product development, potential advantages of Arno’s product candidates, future financial position, future financial results, plans and objectives of management are forward-looking statements. We may not actually achieve these plans, intentions or expectations and Arno cautions investors not to place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. Various important factors that could cause actual results or events to differ materially from the forward-looking statements that we make. Such factors include, among others, risks that the results of clinical trials will not support our claims or beliefs concerning the effectiveness of our product candidates, our ability to finance the development of our product candidates, regulatory risks, and our reliance on third party researchers and other collaborators. Arno is providing this information as of the date of this presentation and does not undertake any obligation to update any forward-looking statements as a result of new information, future events or otherwise.
Arno Investment Highlights

Clinical stage biotechnology company focused on personalized medicine in oncology

- Targeted clinical development pathway for lead compound onapristone
  - Progesterone receptor (PR) positive tumors: Actively enrolling Phase I, first patient in Q1 2014
  - CDx under development to enable patient selection
  - Castration-resistant prostate cancer: Actively enrolling Phase I, first patient in Q1 2014
  - Endometrioid cancer: Phase II planned start Q4 2014, potential orphan indication

- Broad IP position and global patent strategy
  - Key patents projected to expire 2033 - 2035
  - Granted exclusive license for oncology diagnostic technique by University of Minnesota

- Additional pipeline opportunities
  - AR-42, a pan-HDAC with orphan drug designations
  - AR-12, a multi-kinase pathway inhibitor with an impressive pre-clinical dossier

- Raised $30.8 million in October 2013 private placement
  - Led by Soros Fund Management and Perceptive Life Sciences; active participation from management, Board and existing shareholders
  - New investors include OPKO Health, David Bonderman and Dr. Phillip Frost
Management Team & Board of Directors

- **Glenn R. Mattes** *President & Chief Executive Officer*
  - 30+ years of commercialization and healthcare management experience
  - Previously president of Tibotec Therapeutics (a J&J company)
  - Significant experience in oncology, including *Taxotere®* and *Doxil®*

- **Alex Zukiwski, MD** *Chief Medical Officer*
  - 19+ years of global pharma experience
  - Previously, CMO, MedImmune; VP & Head Clinical Oncology, J&J PRD
  - Hoffmann-La Roche, Glaxo Welcome, Rhone Poulenc Rorer
  - Product registration experience: *Taxotere®, Xeloda®, Procrit®, Velcade®, Doxil®*

- **Lawrence A. Kenyon** *Chief Financial Officer*
  - 25+ years of senior-level finance and accounting experience.
  - Previously at Tamir Biotechnology, Inc., PAR Pharmaceutical Companies, Inc. and Neopharm, Inc.

- **Joseph Bisaha, PhD** *VP, Clinical Operations & Project Management*
  - 17+ years pharma product development
  - Previously at Kaizen Clinical Services Inc., ImClone Systems, Inc., Ono Pharma USA Inc., Celgene Corporation and Yamanouchi Pharma America, Inc.

- **David M. Jackson, PhD** *VP, Diagnostics*
  - 16+ years healthcare tech companies and investment firms, specializes in personalized medicine and companion diagnostics
  - Previously at PrimeraDx, Inc., QIAGEN Manchester (formerly Dxs, Ltd.) and Response Genetics

- **Stefan Proniuk, PhD** *VP, Product Development*
  - 16+ years extensive pharma product development
  - Previously at Cima labs

Board of Directors

- **Arie Beldegrun, M.D.** *Chairman*
  - Founder, Exec. Chairman, CEO Kite Pharma
  - Co-founder, Vice Chairman of Board Cougar Biotechnology, sold to J&J
  - Founder of Agensys, Inc., now affiliate of Astellas Pharma Inc.
  - Chair of Urologic Oncology, UCLA

- **Randy Thurman** *Vice Chairman*

- **Glenn Mattes** *Director*

- **William Hamilton** *Director*

- **Tomer Kariv** *Director*

- **Yacov Reizman** *Director*

- **Steven Ruchefsky** *Director*

- **David Tanen** *Director & Secretary*

- **Steven Rubin** *OPKO Board Observer*
Onapristone
Development Program
Lead Candidate Onapristone

Potential to be first approved anti-progestin for oncology indications

- Unique drug development opportunity
  - Oral, type I anti-progestin
  - Anti-tumor activity demonstrated in previous Phase II clinical studies
  - Activated progesterone receptor (APR) as biomarker
    - CDx under development to enable selection of patients most likely to respond
    - R&D collaborations with GE Healthcare, Veridex and Leica Biosystems

- Fill unmet medical needs
- Will be developed for multiple indications
- Broad expression of PR targets in cancer
  - Endometrial / endometrioid
  - Prostate
  - Breast
  - Uterine sarcomas
  - Ovarian

- Will be developed for multiple indications
Onapristone: Recent Accomplishments

<table>
<thead>
<tr>
<th>Month</th>
<th>Event</th>
</tr>
</thead>
</table>
| Sep   | IMPD submission for Ph. I PR+ tumors  
Leica Biosystems CDx collaboration  
2 posters presented ECC |
| Nov   | IMPD submission for Ph. I prostate cancer  
Poster presentation at ECC  
SME designation by EMA |
| Jan   | Enrolled 1st patient in Ph. I trial of onapristone in women with PR+ solid tumors |
| Apr   | 3 posters presented at AACR  
Enrolled 1st patient in Ph. I/II trial of onapristone in men with CRPC |
| Oct   | $30.8 million capital raise  
Debentures converted to common stock, 8:1 reverse stock split  
Leica Biosystems CDx feasibility study complete |
| Dec   | PK data available  
Ph. I PR+ tumor study initiation  
Poster presentation at SABCS |
| Mar   | Granted exclusive license for oncology diagnostic technique by University of Minnesota |
| May   | 2 abstracts selected for poster presentations at ASCO |
Unique Mechanism of Action

Personalized medicine to inhibit growth and proliferation of progesterone receptor driven cancers in both men and women

- Oral type I anti-progestin
- Binds to progesterone receptor (PR), which blocks PR activation
- Prevents PR dimerization
- Subsequently, also blocks processes downstream
  - Prevents phosphorylation
  - Prevents DNA transcription

HIGHLIGHTS OF PRESCRIBING INFORMATION

These highlights do not include all the information needed to use onapristone safely and effectively. See full prescribing information for onapristone.

Onapristone tablets for oral administration

-------------------------------RECENT MAJOR CHANGES-----------------------------------

Indications and Usage, breast cancer(1)

Warnings and Precautions, Deep venous thrombosis(5.2)

-----------------------------INDICATIONS AND USAGE----------------------------------

- Onapristone is an anti-progestin indicated for the treatment of patients with recurrent or metastatic endometrioid cancer which is demonstrated to be activated progesterone receptor positive by an FDA-approved test. This indication is based on response rate. There are no data available demonstrating improvement in patient reported outcomes or survival with onapristone.

- Onapristone is an anti-progestin indicated for the treatment of patients with locally advanced or metastatic prostate cancer which is demonstrated to be activated progesterone receptor positive by an FDA-approved test, after failure of abiraterone acetate.

- Onapristone is an anti-progestin indicated for the treatment of patients with locally advanced or metastatic breast cancer which is demonstrated to be activated progesterone receptor positive by an FDA-approved test, after failure of initial anti-estrogen or aromatase inhibitor treatment. This indication is based on response rate. There are no data available demonstrating improvement in patient reported outcomes or survival with onapristone.
## Prior Clinical Trial Results

<table>
<thead>
<tr>
<th><strong>1st Line Breast Cancer</strong></th>
<th><strong>2nd Line After Tamoxifen Progression</strong></th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Inclusion criteria</strong></td>
<td><strong>Inclusion criteria</strong></td>
</tr>
<tr>
<td>o Post-menopausal women with breast cancer, 1st line endocrine treatment</td>
<td>o Post-menopausal women with breast cancer, progression on prior tamoxifen (28 adjuvant and 90 metastatic patients)</td>
</tr>
<tr>
<td>o Locally advanced disease or elderly patients suitable for endocrine treatment</td>
<td></td>
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<tr>
<td><strong>Protocol</strong></td>
<td><strong>Protocol</strong></td>
</tr>
<tr>
<td>o 100 mg once per day</td>
<td>o 100 mg once per day</td>
</tr>
<tr>
<td>o n=19 enrolled (18 ERpos or PRpos)</td>
<td>o n=118, (102 metastatic disease, 16 locally advanced), 101 evaluable patients</td>
</tr>
<tr>
<td>o Response assessment at 6 months (n=18)</td>
<td>o 53% of subjects ER or PR negative or unknown</td>
</tr>
<tr>
<td>o Serial biopsies obtained</td>
<td>o Response assessment (n=101 evaluable patients)</td>
</tr>
<tr>
<td><strong>Results (Published)</strong>*</td>
<td><strong>Results (Published)</strong>*</td>
</tr>
<tr>
<td>o 10 partial response (PR) (56%), 2 stable disease (SD), 6 PD</td>
<td>o 1 complete response (CR), 9 PR, 39 SD (&gt; 3 months)</td>
</tr>
<tr>
<td>o Median duration of PR or SD: 70 weeks</td>
<td>o Median duration of CR/PR: 11 months</td>
</tr>
<tr>
<td>o Elevated Liver Function Tests (LFTs)</td>
<td>o Median duration of SD: 7 months</td>
</tr>
<tr>
<td>- Grade 2-3 transient, despite continued treatment, mainly observed in the first 6 weeks of treatment</td>
<td>o 20/32 subjects with normal LFTs at baseline had LFT elevation</td>
</tr>
<tr>
<td></td>
<td>- Peaked at 1-2 months and subsequently remained stable or returned to normal with continued treatment</td>
</tr>
</tbody>
</table>

*Robertson, John and Jonat, Walter et al. The Annals of Oncology. 2013*
CDx Development Partnership with Leica Biosystems

Developing companion diagnostic for detection of $\text{APR}^{\text{pos}}$ biomarker to enable selection of patients more likely to respond to onapristone

- Entered co-development agreement with Leica Biosystems in January 2014

- CDx for onapristone, detect $\text{APR}^{\text{pos}}$ cancers
  - Developing IHC in vitro diagnostic (IVD) test used to detect APR in various women's cancers, including endometrioid and breast cancer
  - Will identify patients who’s tumors are APR positive and therefore most likely to respond to treatment with onapristone
  - Automated test
  - Existing tech platform utilized in diagnostic / clinical centers
  - Technology platform for clinical trials

- Establish biomarker-related IP

The PR/APR IHC System
Technique has potential to identify progesterone receptor pathway activation

Gives Arno an additional platform for identifying patients with cancer who would most likely benefit from personalized onapristone therapy

Arno will further develop the PR gene signature as a potentially predictive CDx for anti-progestins, including onapristone

Enables potential of incorporating such a CDx test into ongoing and future clinical studies of onapristone
Examples of the High Definition Test Images
Detection of APR Using Immunohistochemistry (IHC)

Analysis of APR$^{neg}$ and APR$^{pos}$ in tested PR$^{pos}$ endometrioid tumor tissue

Arno sample analysis has shown endometrioid cancer ~45% APR$^{pos}$

PR$^{pos}$/APR$^{neg}$

PR diffused, or evenly distributed, throughout nucleus (no foci) = APR$^{neg}$

PR$^{pos}$/APR$^{pos}$

PR not evenly distributed; distributed in nucleus as aggregates or foci

Foci pattern indicates activation of the PR (APR) = APR$^{pos}$
Detection of APR Using Immunohistochemistry (IHC)

Analysis of APR$^{\text{neg}}$ and APR$^{\text{pos}}$ in tested PR$^{\text{pos}}$ breast cancer tissue

PR$^{\text{pos}}$ / APR$^{\text{neg}}$

PR diffused, or evenly distributed, throughout nucleus (no foci) = APR$^{\text{neg}}$

PR$^{\text{pos}}$ / APR$^{\text{pos}}$

PR not evenly distributed; distributed in nucleus as aggregates or foci

Foci pattern indicates activation of the PR (APR) = APR$^{\text{pos}}$

Arno sample analysis has shown breast cancer ~20-25% APR$^{\text{pos}}$
Onapristone
Clinical Development Program
Initial Development Program Strategy

Target APR^{pos} cancers in two indications with high unmet medical need

- Endometrioid carcinoma
  - Significant percentage are APR^{pos}
  - Potential orphan drug designation
  - Approx. 52,630 new cases of endometrial cancer diagnosed; 8,590 cases of metastatic disease in the U.S.
  - Fewer ongoing clinical trials

- Castration-resistant prostate cancer (CRPC)
  - After failure of abiraterone (ZYTIGA®) or enzalutamide (XTANDI®)
  - Approx. 233,000 new cases of prostate cancer and 29,500 deaths in the U.S. in 2014
  - APR^{pos} percentage remains to be determined, Arno tested limited specimens to date
    - Initial series of CRPC specimens examined showed evidence of APR
    - Data demonstrates PR is present and APR present in a subset of tumors
Active Phase I Trial in PR\textsuperscript{pos} Tumors

- Two-stage study with expansion component
- Stage 1 evaluating six dose cohorts randomized in parallel
- Randomized design of stage 1 expected to accelerate Phase II dose determination by \textasciitilde9 months

**September 2013**
IMPD for CTA submitted to ANSM for Phase I trial

**October 2013**
IMPD approved by ANSM for Phase I trial

**Q1 2014**
Initiation of Phase I trial; actively enrolling
- Women with PR\textsuperscript{pos} tumors (breast, endometrial and others)
- Extended and immediate release formulations
- Up to 6 dose levels 10-50 mg BID and 100 mg QD
- Goal to define recommended Phase II dose and determine overall safety profile

**Q3 2014**
Topline results expected

**May 2014**
Study is 33% enrolled; multiple patients in screening
7 active sites in France
Active Phase I Trial in Advanced CR Prostate Cancer

- Two-stage study with expansion component
- Stage 1 evaluating two randomized dose cohorts
- Randomized design of stage 1 expected to accelerate Phase II dose determination by ~9 months

Q4 2013
IMPD for CTA submitted to UK Health Authority (MHRA) for Phase I trial

Q1 2014
MHRA approved IMPD; actively enrolling separate Phase I dose escalation study
- Men with androgen independent prostate cancer, after failure of abiraterone or enzalutamide
- Tolerance to chemotherapy and various targeted therapies known to be different in males undergoing androgen deprivation treatment

Q2 2014
Commenced active enrollment

Q4 2014
Anticipate ongoing topline results

- Site initiation Feb. 11
- Additional UK sites identified
- First patient enrolled April 2014
Planned Phase II Trial in PR$^{POS}$ Endometrioid Cancer

**Q4 2014**
- Initiate Phase II trial
- Potential orphan drug designation, proposed accelerated approval path to market

**Q4 2014 - 2016**
- Open-label trial, approx. 310 patients in 2 stages
  - **Stage 1**: All comers (~100 patients), correlate APR testing to onapristone response
  - **Stage 2**: Two arms, Primary endpoint ORR, Secondary endpoints include duration of response, watershed plot; for both arms
    - APR Negative: 30 patients
    - APR Positive: 180 patients

**1H 2017**
- Anticipate Phase II data
- NDA & MAA filings

**2014 Highlights**
- ANSM (French Health Authority) meeting March 27
- MHRA (British Health Authority) meeting March 28
- CHMP Scientific Advice meeting planned for July
- FDA meeting planned for August
Onapristone Market Potential

Clear market opportunity for lead indications, given potentially high level of patients with PR positive tumors

<table>
<thead>
<tr>
<th>Indication</th>
<th>Endometrial 1st Line Treatment</th>
<th>CRPC 2nd Line Treatment</th>
<th>Breast 2nd Line Treatment</th>
</tr>
</thead>
<tbody>
<tr>
<td>Total Patient Population - Annually</td>
<td>8,590</td>
<td>30,909</td>
<td>40,215</td>
</tr>
<tr>
<td>Estimated Pr\textsuperscript{pos} Patient Population</td>
<td>6,013</td>
<td>27,818</td>
<td>28,151</td>
</tr>
<tr>
<td>Estimated APR\textsuperscript{pos} Patient Population</td>
<td>2,706</td>
<td>11,127</td>
<td>9,853</td>
</tr>
<tr>
<td>Onapristone Opportunity *</td>
<td>2,165</td>
<td>6,231</td>
<td>7,094</td>
</tr>
</tbody>
</table>

* US Only – 3x for Global Opportunity
**Broad Onapristone Patent Portfolio**

Robust patent portfolio and IP pipeline

<table>
<thead>
<tr>
<th>Patent Description</th>
<th>Details</th>
<th>Type</th>
<th>Submission Date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Identification of APR predicts anti-progestin activity</td>
<td>Diagnostic used to identify patients most likely to respond to onapristone and other anti-progestins</td>
<td>Method of Use</td>
<td>Oct 2011: Provisional patent application filed</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Oct 2012: Expanded US utility and PCT application filed</td>
</tr>
<tr>
<td>Polymorph</td>
<td>Patent describing and protecting the crystal forms of the drug substance and those crystal forms used to make drug product</td>
<td>Utility</td>
<td>Filed in March 2013</td>
</tr>
</tbody>
</table>

- Two applications pending approval; five applications expected to file in 1H 2014
- Projected expirations, 2033 - 2035
- Planned future patents for process, formulation and utility
- Additional patents intended for submission over next 24 months should result in enhanced freedom to operate
Additional Pipeline Candidates
Additional Pipeline Candidates: AR-42 and AR-12

Exclusive development & commercialization rights to novel oncology therapies through license from Ohio State University

AR-42

Oral, broad spectrum Pan-DAC inhibitor in solid tumors and hematological malignancies

- Ongoing Phase I/IIa in collaboration with OSU
  - RP2D determined
  - Expansion phase initiated
- Initiated Phase I study in combination with decitabine in 3Q13
- Orphan designations in US and EU
  - US: CNS schwannoma, meningioma
  - EU: schwannoma, meningioma, NF-2

AR-12

Potential first-in-class PDK-1 inhibitor with demonstrated preclinical efficacy in wide range of tumor types

- Completed stage I of two-stage, multi-site trial
  - Further clinical development planned before proceeding
  - Improved formulation shown to substantially increase bioavailability in preclinical models
  - Preclinical data for AR-12 use in infectious disease
Arno Financials & Milestones
October 2013 Recapitalization Highlights

Focused on driving development of onapristone in most efficient and cost-effective manner

- $30.8 million gross proceeds
  - Funding for two Phase I trials and initiation of Phase II
- 20.4 million shares of common stock outstanding as of May 15, 2014
  - Post equity offering, debenture conversion and 1:8 reverse split
- Options and warrants to purchase 55.9 million shares as of Mar. 31, 2014
- ~$41 million market capitalization as of May 19, 2014

- Well-regarded investors endorse the development program
  - Existing: Soros Fund Management, Perceptive Advisors, Sabby Capital, The Pontifex Group, Commercial Street Capital, and Bellco Capital
  - New: OPKO Health, Mr. David Bonderman (founding partner of TPG Capital), Dr. Phillip Frost (Chairman and CEO of OPKO) and Deerfield Management
- OPKO Health granted right of first negotiation and right to designate one observer to Board of Directors
## Current Financials

### Balance Sheet as of March 31, 2014

<p>| | |</p>
<table>
<thead>
<tr>
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</thead>
<tbody>
<tr>
<td>Cash and Cash Equivalents</td>
<td>~$21.4 million</td>
</tr>
<tr>
<td>Total debt</td>
<td>$0</td>
</tr>
<tr>
<td>Shares outstanding</td>
<td>~20.4 million</td>
</tr>
<tr>
<td>Warrants</td>
<td>~48.0 million</td>
</tr>
<tr>
<td>• Weighted average exercise price - $2.65</td>
<td></td>
</tr>
<tr>
<td>• Expiration – 10/31/14 – 10/29/18</td>
<td></td>
</tr>
<tr>
<td>Stock Options</td>
<td>~7.9 million (~1.4 million exercisable)</td>
</tr>
<tr>
<td>• Weighted average exercise price - $2.71 ($3.51 for exercisable options)</td>
<td></td>
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</tbody>
</table>
Onapristone Recent & Anticipated Milestones

Nov 2013
Prostate cancer Ph. I IMPD submission

Jan 2014
CDx co-development agreement with Leica Biosystems

Apr 2014
Prostate cancer Ph. I study start

Q4 2013
PK data available, Ph. I PR+ tumor study initiation

Q4 2014
Initiate Ph. II PR+ endometrioid cancer trial for accelerated approval

Q4 2014
Anticipate ongoing Prostate cancer Ph. I data

Q2/3 2015
Topline results expected from Phase I PR+ trial

Q4 2015
Interim data from the Ph. II PR+ endometrioid cancer study

Q4 2016
Ph. II PR+ endometrioid cancer study data base closure

1H 2017
Anticipate Ph. II Data File NDA & MAA endometrioid cancer

Aug/Sept 2013
PK start, Ph. I PR+ tumors IMPD submission

Q4 2013
PK data available, Ph. I PR+ tumor study initiation

3Q 2014
Topline results expected from Phase I PR+ trial

Q2/3 2015
Interim data from the Ph. II PR+ endometrioid cancer study

2013
2014
2015
2016
2017
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