



# Personalizing Immunotherapy for Cancer and Infectious Disease

June 2014

# Forward Looking Statements

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# Personalizing Immunotherapy for Cancer and Infectious Disease



- **AGS-003: phase 3 trial in mRCC under SPA expected to be fully enrolled in early 2015**
  - In phase 2 combination trial, median OS 2X greater and long-term survival ( $\geq 30$  months) 4X greater than targeted therapies alone\*
- **AGS-004: phase 2b trial in HIV fully funded by \$39.3M NIH contract with data expected in mid-2014**
- **Arcelis™: fully personalized immunotherapy technology platform that is potentially applicable to a wide range of cancers and infectious diseases**
  - Overcomes disease-induced immunosuppression to induce memory T cells known to correlate with improved clinical outcomes in cancer and HIV

\* Compared to data from independent databases of similar patients

# Strategy Overview



- **AGS-003: potential first-line standard of care in mRCC in combination with approved therapies**
  - Interim data analyses expected in 2015 and final OS data expected in mid-2016
  - Multiple phase 2 trials in other indications planned for this year
- **AGS-004: potential for HIV eradication, elimination of antiretroviral therapy (ART)**
  - Two phase 2 trials planned for this year
- **Arcelis: automated, centralized manufacturing process developed**
  - Plan to build one commercial facility to serve all of North America
  - Cost of goods expected to be comparable to typical biologics

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# Large Biopharma Companies Focused on Cancer Immunotherapy

- Roche, Merck, BMS and others developing checkpoint inhibitors for multiple solid tumors
- Novartis developing personalized T cell therapy for leukemia
- Pfizer, J&J collaborating with MD Anderson for novel immunotherapies

**Arcelis is designed to provide key advantages over other approaches**





## Arcelis Technology Platform:

fully personalized mRNA-loaded dendritic cell immunotherapy based on work of Nobel Prize-winning, Co-founder Ralph Steinman

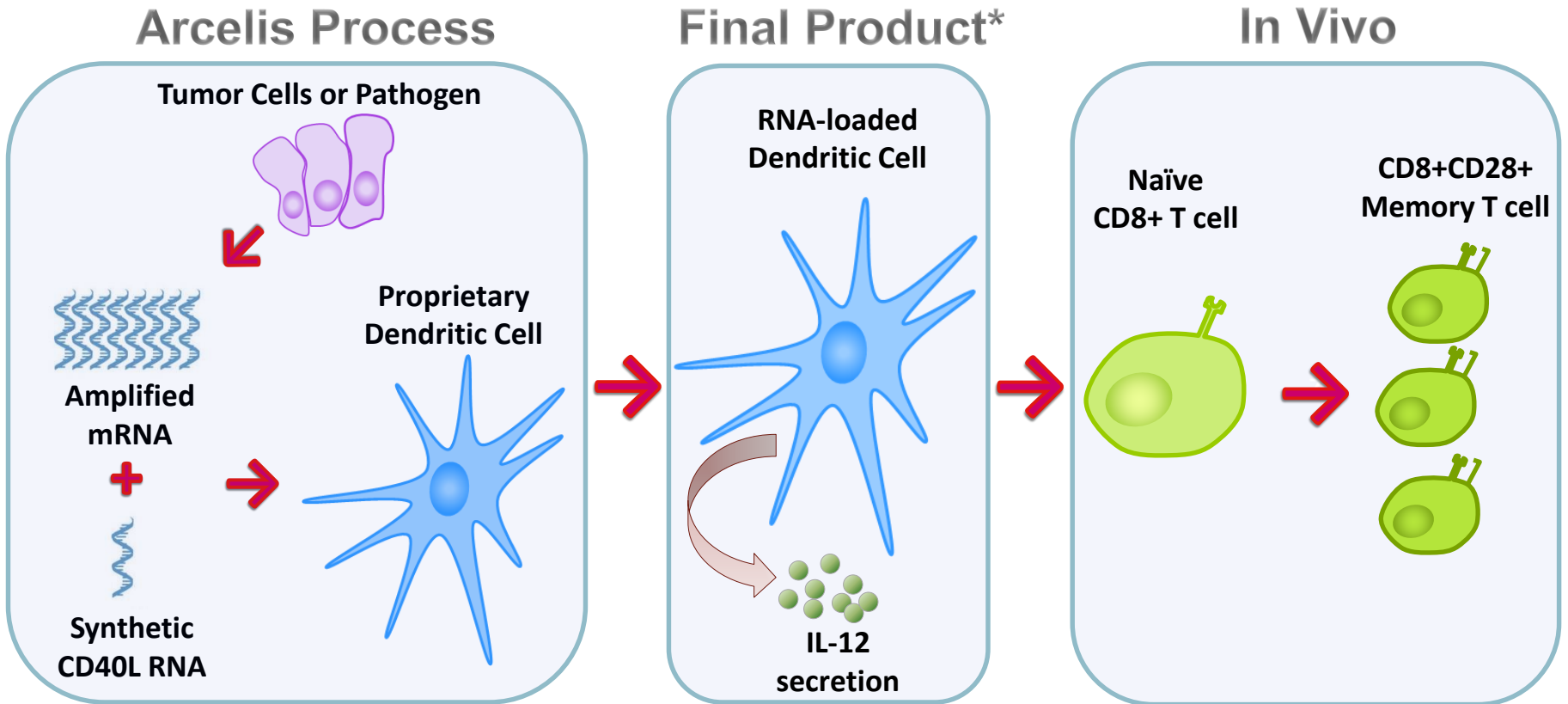
# Arcelis is Designed to Contain the Key Attributes Necessary for an Effective Immunotherapy



- Target patients' disease-specific antigens, including mutated antigens
- Overcome the immune suppression that exists in cancer and infectious disease patients
- Induce memory T cells independent of PD-1 checkpoint inhibition
- Minimal toxicity allows combinations with other therapies
- Cost-effective, scalable, automated centralized manufacturing

**Potentially applicable to a wide range of cancers and infectious diseases**

# Arcelis Products Induce Patient-specific Memory T cells



\* Delivered by intradermal injection





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AGS-003  
Development  
in mRCC

# Significant Unmet Need in mRCC

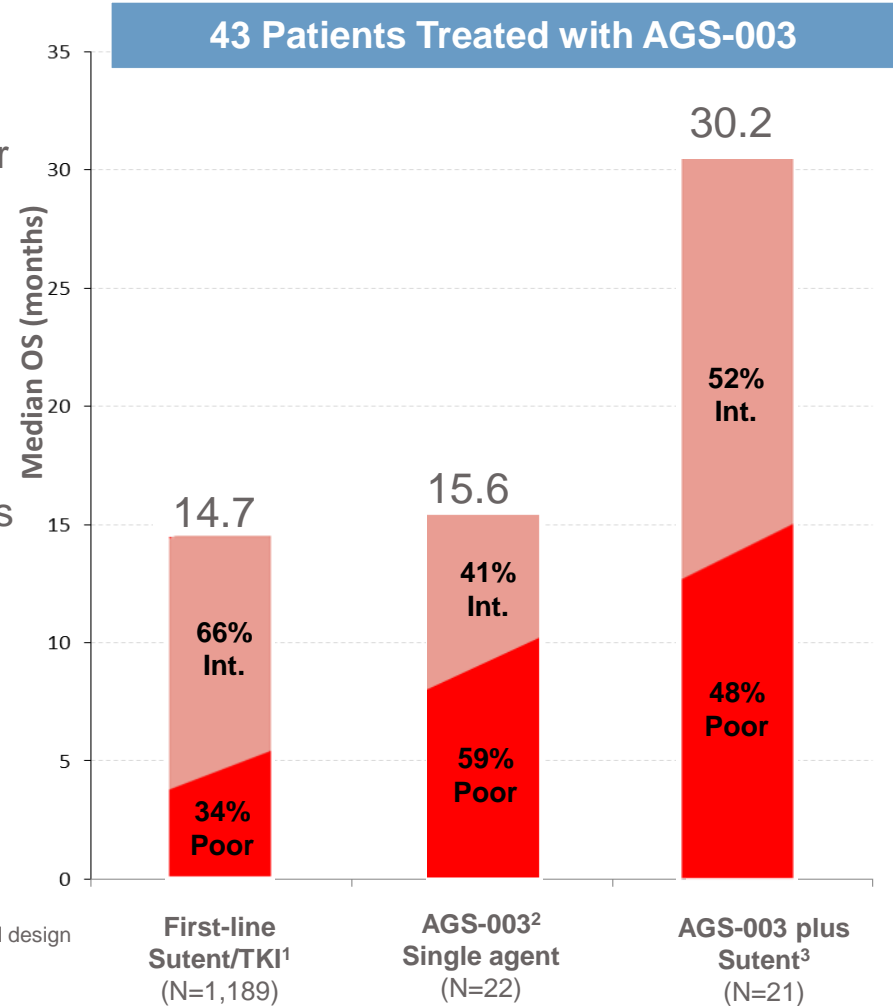


- **Large addressable market opportunity**
  - Worldwide mRCC market is estimated at > \$2 billion
  - 20,000 - 25,000 new mRCC cases/year estimated in U.S. alone
- **Limited effectiveness of Sutent and other targeted therapies (TKIs) in intermediate and poor risk mRCC patients**
  - Median overall survival is less than 15 months
- **Overlapping toxicities of current therapies prevent their use in combination**

# AGS-003 in Combination with Sutent Doubled OS

- **Single agent AGS-003 phase 1/2 trial**
  - Median OS comparable to TKIs despite higher percentage of poor risk patients
    - Only 3 patients received TKI 2nd line therapy
- **AGS-003 + Sutent phase 2 trial**
  - Median OS > 2X increase compared to TKIs despite higher percentage of poor risk patients
    - Greater benefit in intermediate risk patients
      - Median OS intermediate risk: 57.1 months

**Phase 3 patient population expected to be ≥ 65% intermediate risk**



1. Ko JJ, Choueiri TK, Rini BI et al. First-, second-, third-line therapy for mRCC: Benchmarks for trial design from the IMDC. Br J Cancer. 1 April 2014;1-6.

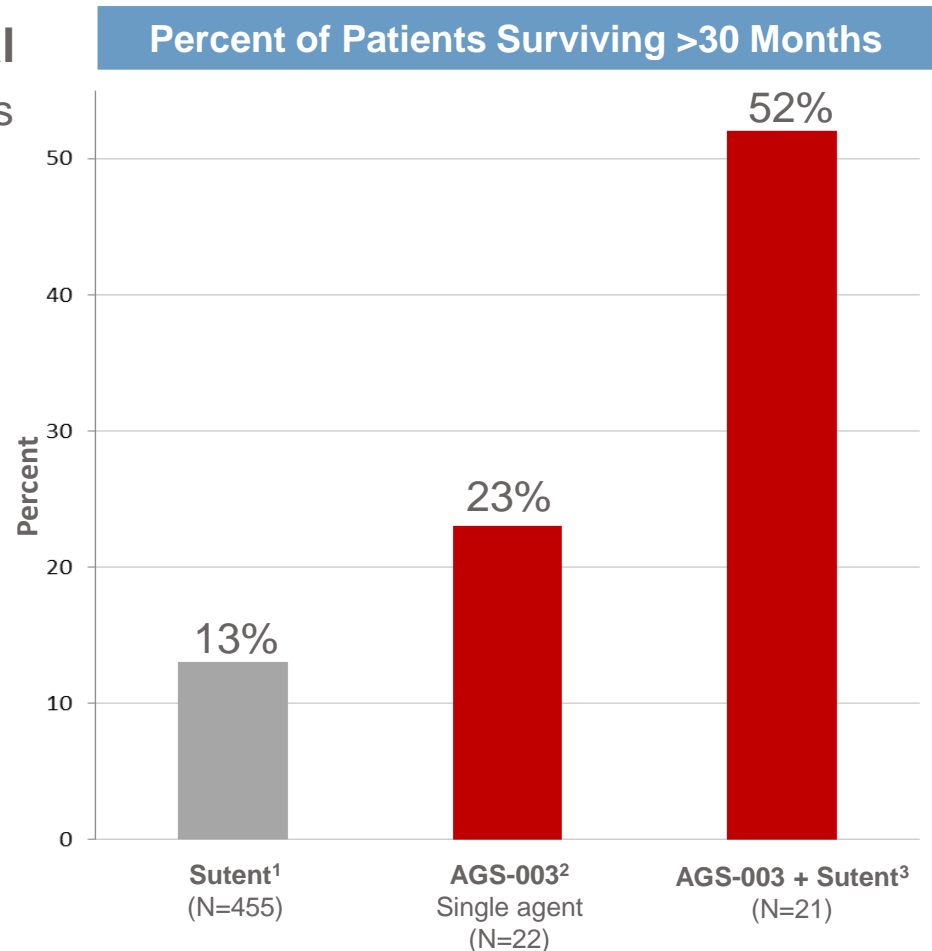
2. Data on file at Argos Therapeutics. September 2013.

3. Amin A et al. J Clin Oncol. 2014;32:5s (suppl; abstr 4524).

# AGS-003 in Combination with Sutent 4X the Frequency of Long-term Survivors



- **Single agent AGS-003 phase 1/2 trial**
  - ~2X the frequency of long-term survivors compared to Sutent trials
- **AGS-003 + Sutent phase 2 trial**
  - 4X the frequency of long-term survivors compared to Sutent trials
  - 7/21 survived > 4.5 yrs
  - 5 survived > 5 yrs
  - 2 remain in remission



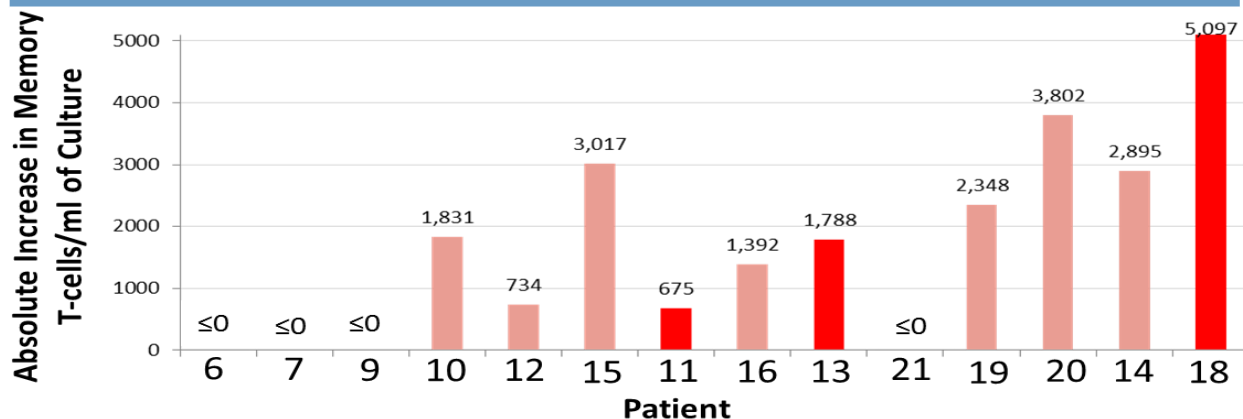
1. Motzer et al. British Journal of Cancer (2013) 108:2470–2477

2. Data on file at Argos Therapeutics. September 2013.

3. Amin A et al. J Clin Oncol. 2014;32:5s (suppl; abstr 4524).

# Established Correlation between Immune Response and Survival\*

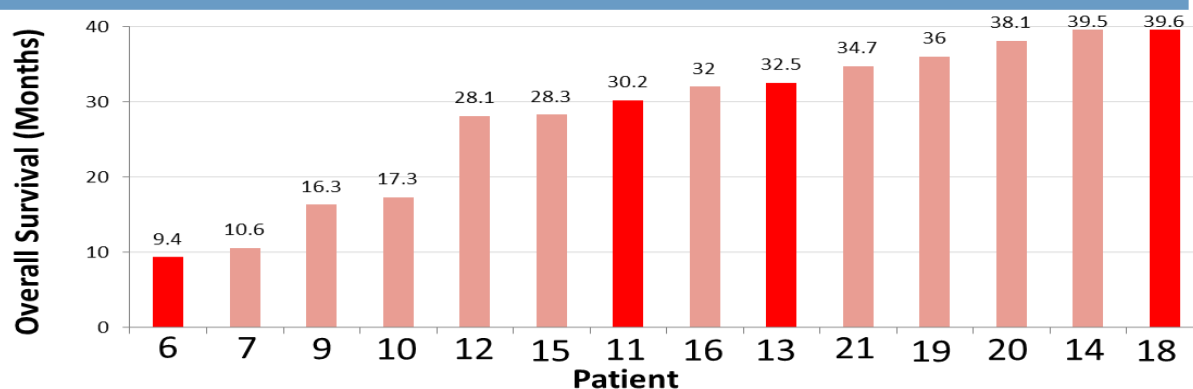
Increase in Tumor-specific Memory T cells: Prior to 1<sup>st</sup> dose vs. after 5<sup>th</sup> dose



■ Poor Risk  
■ Intermediate Risk

Immune response also correlated with PFS and tumor response

Overall Survival from Initiation of Treatment as of May 2012



\*AGS-003 + Sutent phase 2 trial: 14/21 patients received ≥ 5 doses and were evaluable

Only immunotherapy with statistically significant correlation between magnitude of immune response and duration of survival (Spearman's  $\rho=.8$ ;  $p\leq.002$ )

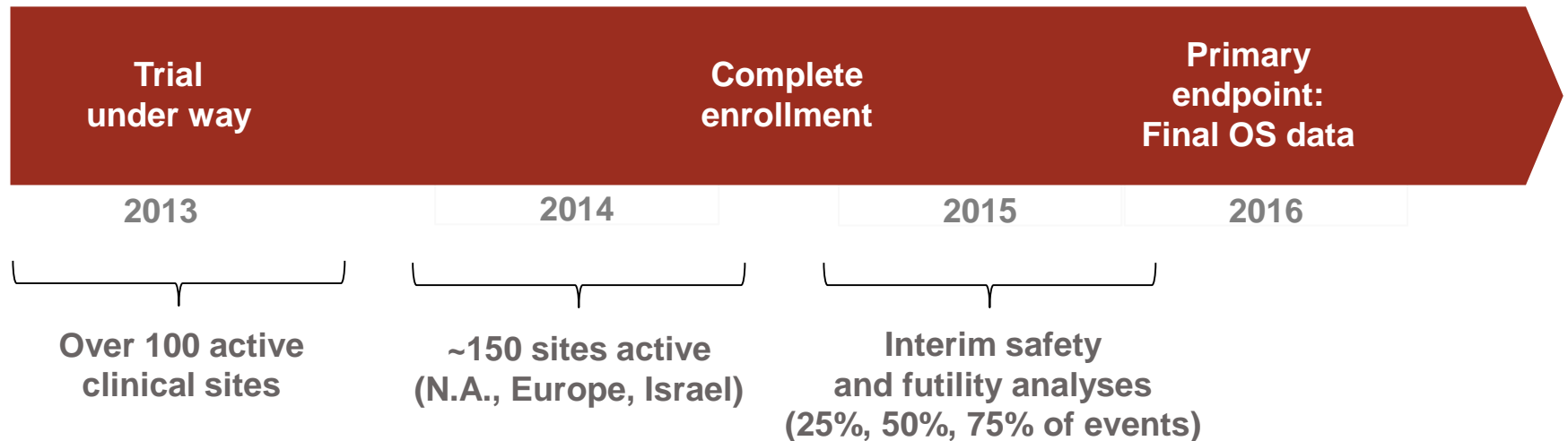
# Phase 3 ADAPT Trial Design

- 450 newly diagnosed, intermediate and poor risk mRCC patients
  - Open label, first-line treatment
- Similar design as phase 2 combination trial with key enhancements
  - AGS-003 dosing continues through initial progression
  - Intermediate and poor risk patients with 1-4 risk factors (phase 3 patient population is currently ~70% intermediate risk)
- Primary endpoint ~6 month OS improvement (.708 hazard ratio)
- Granted SPA by FDA and received Fast Track designation



\* Standard therapy initiates with Sutent. Other therapies may be substituted for Sutent intolerance or progression.

# ADAPT Phase 3 Trial Key Milestones



**No competitive product trials currently enrolling**



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AGS-004  
Development  
in HIV



# AGS-004: Pursuing HIV Eradication and Elimination of ART



- **Key features**

- Utilizes identical process and dendritic cells as AGS-003
- Use of patient's mRNA captures diversity of patient's virus
- Overcomes immune defects associated with HIV infection
  - Generates memory T cells without CD4+ T cell help

- **Development status**

- Completed phase 1 and phase 2a trials
  - First HIV immunotherapy to demonstrate sustained reduction in viral load
- Ongoing phase 2b trial is fully funded by \$39.3M NIH contract
- Two phase 2 trials for HIV eradication and elimination of ART planned for 2014

# AGS-004 Phase 2b Trial Data Expected Mid-2014



- Randomized 2:1, double-blinded, placebo-controlled
- Primary endpoint:
  - $\geq 1.1$  Log reduction in viral load after 12 weeks of ART interruption (AGS-004 vs. placebo)
- Enrollment completed (N=53)
- Positive data in mid-2014 would provide proof of concept for treatment aimed at eradication and elimination of ART

**Trial is fully funded by \$39.3M NIH contract**

# Two AGS-004 Phase 2a Trials to Initiate in 2014

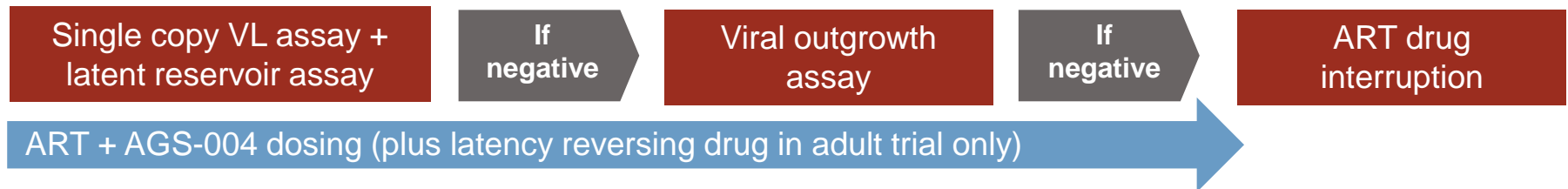


- **Adult trial<sup>1</sup>**

- Rationale: latency reversing drug exposes reservoir to immune system
- Endpoint: virus eradication
- Collaboratory of AIDS Researchers for Eradication, or CARE, will fund costs of trial, except manufacturing

- **Pediatric trial<sup>2</sup>**

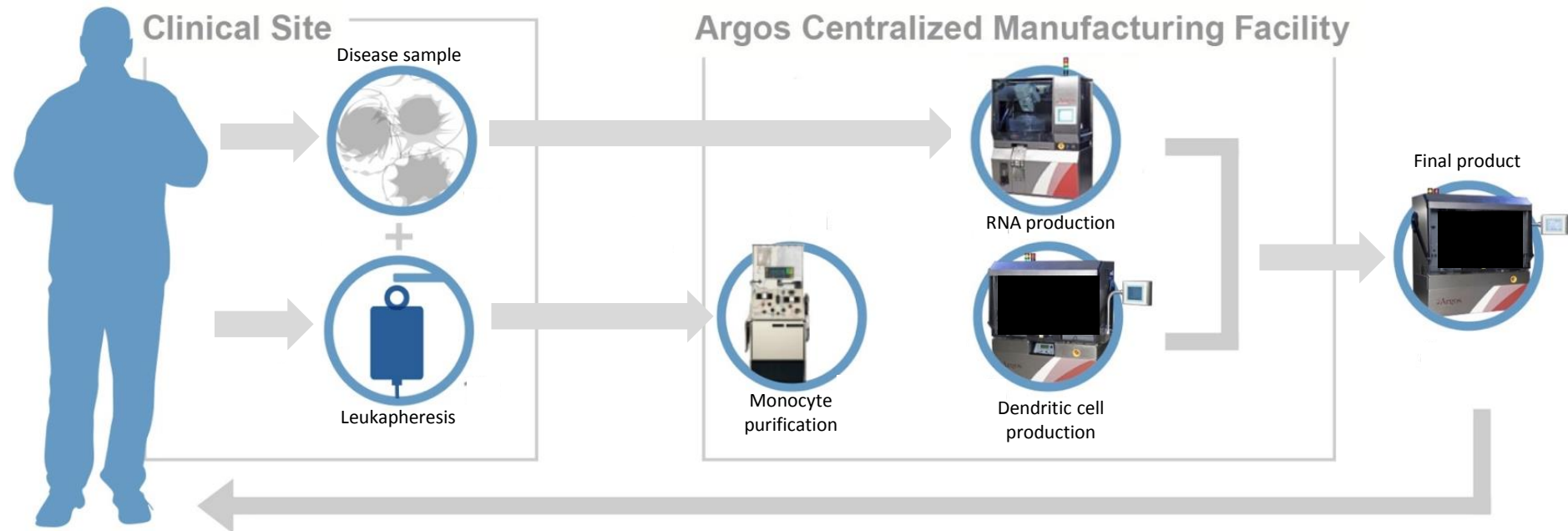
- Rationale: resemble individuals who can control virus without ART except no anti-viral memory T cells
- Endpoint: elimination of ART



1. Trial to be led by David Margolis, University of North Carolina

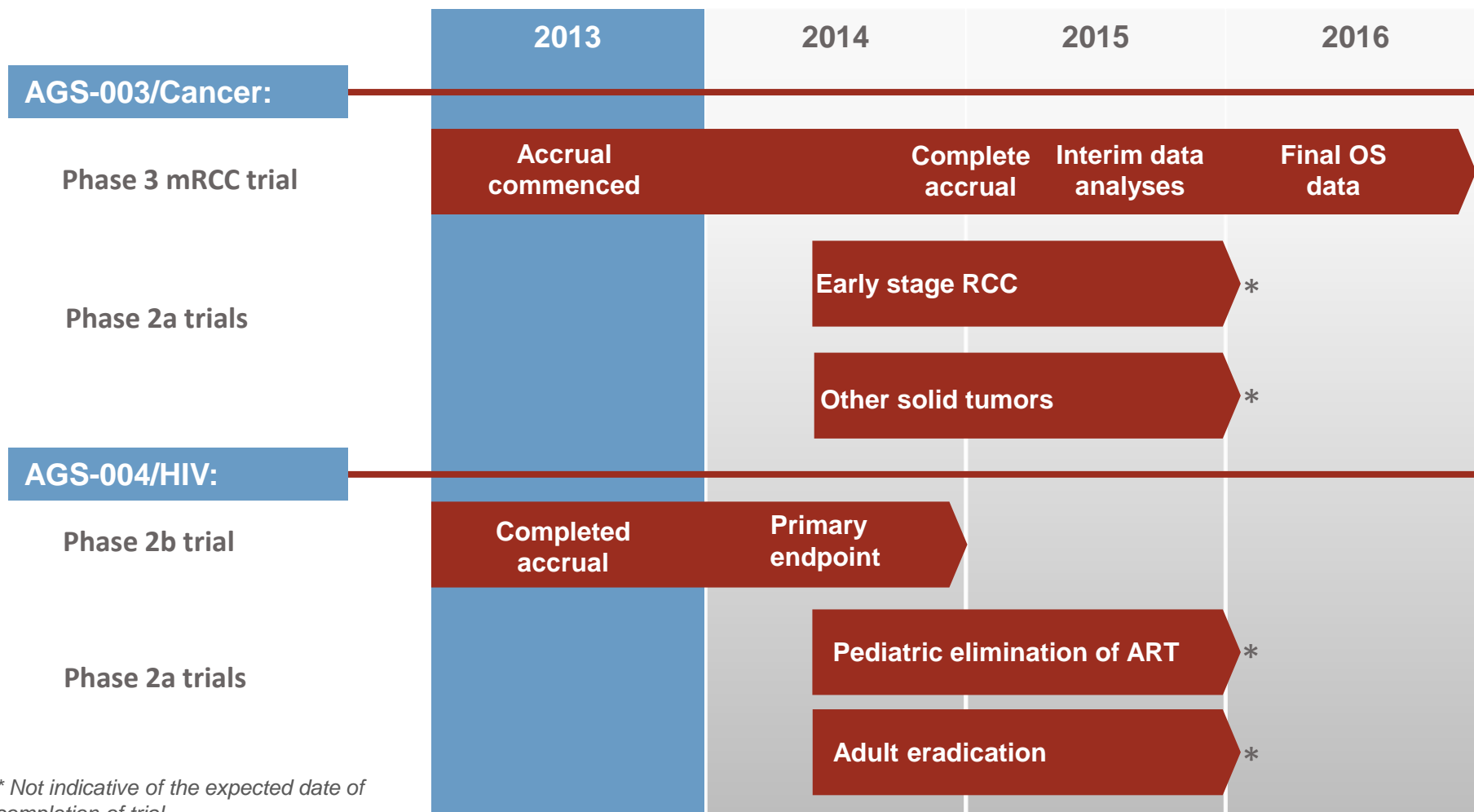
2. Trial to be led by Katherine Luzuriaga, University of Massachusetts, and Deborah Persaud, Johns Hopkins University

# Arcelis Process Automation is Key to Commercial Success



- Single disease sample and leukapheresis provide multiple years of therapy
- One facility can serve all of North America
- Cost of goods expected to be comparable to typical biologics
- Protected by 12 U.S. patents, 8 U.S. patent applications and ~60 foreign counterparts

# Pipeline / Milestones



\* Not indicative of the expected date of completion of trial

# Recently Completed IPO Expected to Fund Through Major Value Inflection Point



- **Cash on hand expected to fund:**
  - AGS-003 pivotal phase 3 mRCC trial through data
  - AGS-003 phase 2 trials in early stage RCC and other solid tumors
  - AGS-004 phase 2 trials in HIV (pediatric elimination of ART; adult eradication)
    - To the extent not covered by non-dilutive financing
  - Initiation of planned lease, build-out and equipping of automated commercial manufacturing facility
    - Pursuing various non-dilutive financing options
- **AGS-004 ongoing phase 2b HIV trial is fully funded by NIH**

**Plan to be ready to commercialize independently following FDA approval**

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- **Arcelis™: fully personalized immunotherapy technology platform**
  - Overcomes disease-induced immunosuppression
  - Induces memory T cells known to correlate with good clinical outcomes
  - Potentially applicable to a wide range of cancers and infectious diseases
  - Automated centralized manufacturing process

\* Compared to data from independent databases of similar patients