

# Validation & Drug Development RFAs

## Enhancing Translational Impact of VA Research

### Informational Webinar

Arun J Sharma, PhD  
[Arun.Sharma@va.gov](mailto:Arun.Sharma@va.gov)

BLR&D  
ORD

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# ORD Strategic Priority 2



## Substantial Real-World Impact

**Deploy our research resources to deliver meaningful outcomes, ranging from validated knowledge generation to policies to products.**

- **Shift from an individual award focus to the series of steps necessary to achieve a desired outcome (e.g., roadmaps).**
- **Translate VA inventions into licensed products.**
- **More rapidly implement effective interventions into routine care**

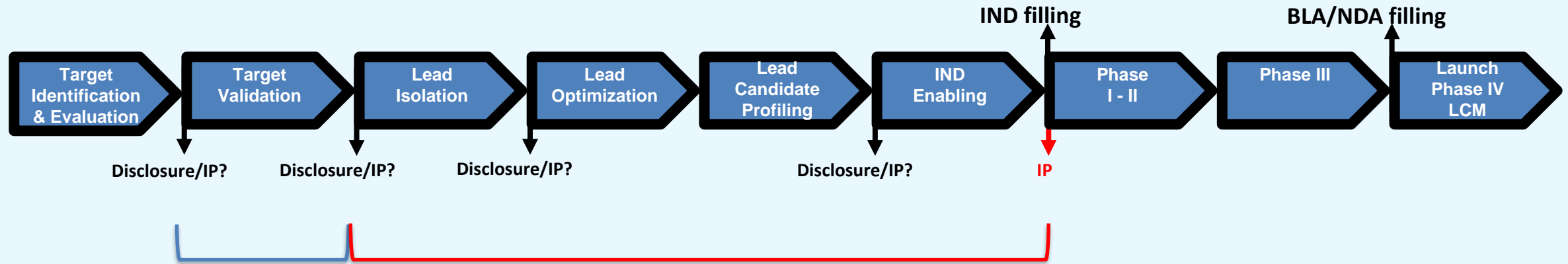
# Agenda

- **RFAs to Enhance Real-World Impact of VA research**
- **Requirements & Evaluation Criteria for Validation RFA Proposals**
- **Changes to the Drug Development RFA**
- **Questions/ Comments**

# Validation & Drug Development RFAs: Objectives

- **To accelerate translational impact of VA research on the clinical care of Veterans.**
- **To support advancement of basic research findings from early discovery stage to regulatory submissions (IND/IDE), with an eventual goal of expediting the delivery of novel therapeutic products for the clinical care of Veterans.**

# Enabling Drug Development Studies



**RFA**      **Validation Studies**      **Lead Isolation & Optimization and Pre-IND studies of Drugs and Biologics**

**Proposals should have clearly defined Milestones and Go/No-Go decision points.**

**Successful outcome from proposals should move the projects from one stage of drug development to the next stage.**

# Validation RFA: Primary Research Focus

**Veteran related health topics responsive to this announcement include, but are not limited to:**

- Long- and short-term consequences of military environmental exposures (e.g., burn pits) relevant to deployment in Iraq and Afghanistan
- Spinal cord injury (SCI)
- Traumatic brain injury (TBI)
- Polytrauma
- Post-traumatic epilepsy
- Vision and/or hearing deficits related to blast injury
- Burns
- Fracture Repair
- Chronic pain related to neurotrauma
- Wound Healing
- Mood and anxiety disorders (including depression, PTSD, acute stress disorder, etc.)

# Validation RFA: Essential Requirements for the Proposals

- **Validate results from a rigorously conducted scientific study.**
- **Lead to translational studies capable of improving the clinical care of Veterans.**
- **De-risk the future decisions to proceed with additional drug development studies, IND/IDE-enabling studies, and clinical trials.**
- **Submit an invention disclosure form to VA-Technology Transfer Program for proposals that may result in new inventions.**

# Studies that are appropriate for this RFA

1. Replicate published findings from an animal model of a disease condition to another model of the same disease/condition important to Veterans health.
  - a. RFA is not for validation of small unpublished studies or for mechanistic studies of unpublished or published findings.
2. Replicate published or unpublished “omic” findings important to Veteran health from one large population in another large population.
  - a. RFA only supports funding studies involving existing data sets and samples.
3. Validate novel therapeutic targets in another model of the same human disease/conditions.
  - a. Target from an unpublished study should have a clear translational and therapeutic potential.
4. Develop & validate a new animal model for a Veteran health condition that is lacking an effective animal model.
  - a. RFA will also accept proposals to replace an existing primate-, canine- or feline-model for a Veteran health condition.
5. Develop & Validate tissue culture models of diseases to replace currently used animal models.



# Studies that are not appropriate for this RFA

- Hypothesis driven validation of preliminary results.
- Studies extending findings from one disease/condition to another disease/condition.
- Studies unable to de-risk the future decisions to proceed with additional drug development studies, IND/IDE-enabling studies, clinical trials, or clinical care Veterans.
- Merit award applications that could be submitted under other RFAs, including those from investigators lacking BLRD eligibility\*.
- Merit award applications that could be submitted under other RFAs from investigators who wish to access the higher budget cap of this RFA or circumvent one active award/RFA rule.

\*Non-clinician PD/PIs are not required to apply for eligibility for this RFA but must have a 5/8ths VA paid appointment at the time of application, a promise of employment after funding or WOC appointment would not be sufficient qualification to apply for this RFA. **The funding under the Validation RFA does not confirm eligibility to submit applications to other RFA.**

# Validation RFA: Key Evaluation Criteria

- Is the proposal addressing a critical health care need of Veterans?
- Is the proposal based on a rigorously conducted scientific study?
- Whether outcome will lead to significant improvements over existing approaches?
- Are there other approaches that negates or minimizes the need for conducting this study?
- Is there a need for Validation? Is the study addressing that issue?
- Is the selection of the validation model appropriate and will it provide independent means of validating the findings?
- Would the results of this study de-risk future clinical development process or clinical care?
- After validation will the project progress to the next stage of clinical development?
- Would validation of new animal or cell culture model reduce the use of existing animal models?
- Are the milestones, timelines, and Go/No-Go criteria appropriate?

# Validation RFA LOI Requirements

- Submission of Validation Merit Review applications require an approved Letter of Intent (LOI).
- Please review the RFA and the LOI guidance and follow the instructions.
- Determine whether the proposal meets the BLR&D purview and the scope of the Validation RFA.
- Determine whether the proposal meets the essential requirements of the Validation RFA.
- Ensure that the proposal satisfies the evaluation criteria of the Validation RFA.

Use the LOI to address the issues identified above when responding to different LOI Sections:

Section 1: Provide information on the results you want to validate.

Section 2: Show that the finding to be validated is based on a rigorously conducted study.

Section 3: Briefly describe the results of the previous study and its importance.

Section 4: What is the need/rationale for validating the finding, and how the results will improve future development of this technology and health care of Veterans.

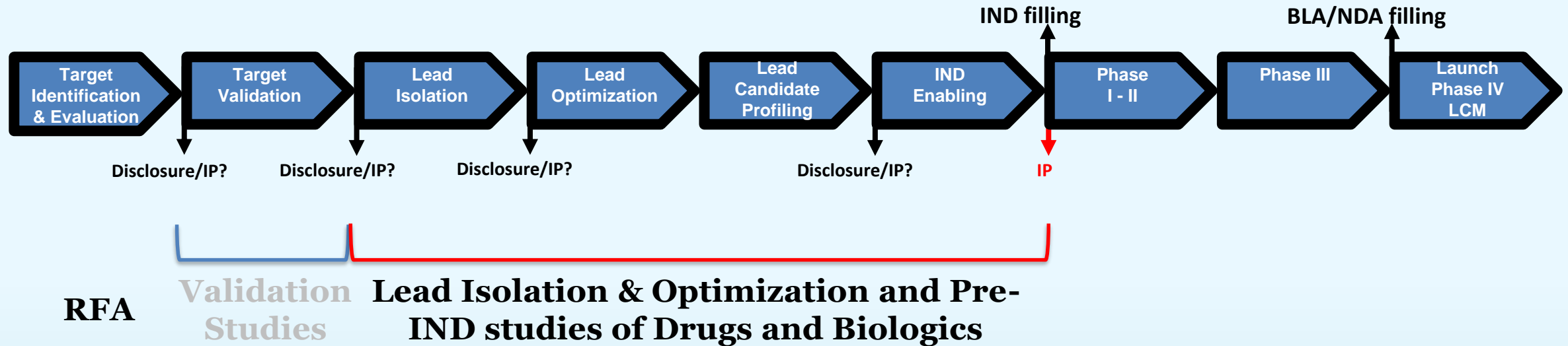
Section 5: Describe the experimental approaches and analyses to be used for validation study & Go/No-Go decisions points.



# SCOPE OF DRUG DEVELOPMENT RFA BX-20-010 HAS BEEN EXPANDED



# A Key Change to the Drug Development RFA



**RFA will support lead isolation and optimization for a validated novel therapeutic target with potential to address an unmet clinical need of Veterans.**

**PI should have established a high-throughput assay with appropriate controls, and have defined criteria for lead selection, optimization and validation.**

**Successful outcome of the study should move the lead candidate to IND-enabling studies.**

# Summary

- New RFAs have been announced to enhance translational impact of VA research on Veterans health.
  - RFAs may be found on the VA intranet: <http://vaww.research.va.gov/funding/rfa.cfm>
- Guidance for Validation RFA has been updated to define:
  - Applications Requirements
  - Review Criteria
  - Expected Outcome
  - Eligibility Changes
- Drug Development RFA will support lead isolation, characterization & validation.
- Proposals for Drug Development BX-20-010 & Validation BX-20-13 RFAs require an approved LOI; LOIs are due on November 1, 2019.
  - Information on the LOIs can be found on BLRD/CSRD Resource Page: [https://www.research.va.gov/services/shared\\_docs/resources.cfm](https://www.research.va.gov/services/shared_docs/resources.cfm)

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**For additional questions & comments please contact:**

**[VHABLRD-CSR@va.gov](mailto:VHABLRD-CSR@va.gov)**