Webinar on Enhancing Translational Impact of VA Research: BX-20-010: *BLRD Merit Review Award for Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics* and BX-20-013*: BLRD Merit Review Award for Validation of Studies of Importance to Veteran Health*

Frequently Asked Questions

Do the RFAs just allow for device development?

Both the BX-20-010*: BLRD Merit Review Award for Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics* and BX-20-013*: BLRD Merit Review Award for Validation of Studies of Importance to Veteran Health* do allow for applications to be submitted with a focus on device development, as long as the application focus falls within the BLR&D purview. Generally, most devices fall in the purview of other VA-ORD Services.

Are novel diagnostics targeted toward personalized therapeutics relevant and, if so, for which mechanism?

Application focused on novel diagnostics are accepted under both BX-20-010 and BX-20-013 RFAs, depending on the proposed study. If the application seeks to validate a novel diagnostic biomarker identified in one large cohort, into another independent cohort, then the application will be appropriate for the RFA BX-20-013: *BLRD Merit Review Award for Validation of Studies of Importance to Veteran Health*. However, if the application aims to address some of the FDA requirements then it might be appropriate for the BX-20-010 RFA: *BLRD Merit Review Award for Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics*. Please reach out to your SPM to discuss the application and to determine which RFA is appropriate.

Is there a special review panel for the RFA applications or they go to regular review panels depending on the research fields?

Applications submitted under these RFAs will be reviewed by the subject matter panels. However, the reviewers will be instructed to follow the RFA specific guidance given to them and review the applications according to the specific criteria mentioned in the RFAs.

Additionally, at the funding decision meetings, applications submitted to these RFAs will have additional considerations from the Merit Award applications for funding decisions.

If a PI submits more than one application with similar subject matter to different BLRD and CSRD RFAs, the applications may be assigned to the same review panel.

Is a current holder of a BLRD Merit eligible for either RFA?

Yes, if the BLRD award is for an application submitted to a different RFA.

The eligibility requirements for the two RFAs differ. BX-20-010 RFA: *BLRD Merit Review Award for Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics* requires prior approval for non-clinician eligibility. There is no prior approval requirement for non-clinician eligibility. RFA BX-20-013: *BLRD Merit Review Award for Validation of Studies of Importance to Veteran Health* requires that a PI have a paid VA appointment of 5/8th at time of application submission.

How many awards do you hope to fund? Total scope of program?

There are no specific set-aside funds for awards resulting from application submission to these RFAs. However, the type of applications submitted to these RFAs are a high priority for the Director of BLRD and for the Chief Research and Development Officer of the VA Office of Research and Development.

Applications submitted to these RFAs will have additional considerations from the Merit Award applications for funding decisions.

Are both RFAs appropriate to advance a drug or biologic that has been licensed to a pharmaceutical company (similar to SBIR), if the target is beyond the current targets being explored by drug company?

If the invention has already been licensed to a pharmaceutical company, please contact your SPM and Technology Transfer Program coordinator to explain the requirement for the support and potential benefit to Veterans health and VA's intellectual property rights. One of the major objectives of these RFAs is to make VA inventions attractive to pharmaceutical or biotechnology companies for licensing. We expect that the pharmaceutical or biotechnology company will take the lead on the further development of the invention.

Will toxicity and pharmacokinetic studies fall within RFA BX-20-10? How about GMP studies?

Toxicity and pharmacokinetic studies fall within the purview of the *Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics*, BX-20-010. Support for GMP studies will be evaluated on case-by-case basis, it is expected that by the time GMP studies are initiated, the invention has been licensed or has a potential licensee to share the cost of the study.

May RFA BX-20-010 be used to support studies to repurpose an already FDA-approved drug to a new population and/or for a new purpose for which it is not currently approved?

No, the *Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics*, RFA BX-20-010, does not support generation of results to support repurposing an already FDA-approved drug. However, if strong data demonstrates that a drug can be repurposed, the RFA will support validation of those results to de-risk future clinical development.

Is this RFA, BX-20-010: Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics appropriate for a novel drug that is being developed for one disease but may be applicable to a different disease in a different organ? and this novel drug has already been disclosed to VATTP.

If the investigator has results demonstrating a new role or function for an existing drug, a study may be proposed to validate this finding in order to de-risk future clinical development. The RFA does not support a project where the goal is to generate new data demonstrating the novel function of a drug.

May RFA BX-20-010 be used for studies that combine two or more FDA-approved drugs into a single compound or for simultaneous use?

No, RFA BX-20-010, *Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics*, only supports studies that have rigorously conducted scientific data supporting the advantages of drug combination for a study to de-risk future development for clinical care.

Is this RFA BX-20-010 appropriate for experiments looking for an optimal dose range, upper limit of toxicity, or minimal effective dose in an animal model?

Yes, if the outcome of the study will lead to IND filling or other IND-enabling study, RFA BX-20-010: *Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics*, may be appropriate for such studies. Additionally, the application should be based on rigorous scientific findings and a clear expectation to improve the clinical care of Veterans.

There is not an RFA for Drug Development* on the RFA and Program Announcement website*?

What is often referred to as the Drug Development RFA is really called *Lead Isolation and Optimization and Pre-IND studies of Drugs and Biologics,* RFA BX-20-010.

For RFA BX-20-013, do the previous studies that the proposed application build upon need to be accomplished by the applicant? Or can we take a published study by another group and move it forward (with new animal model, etc.).?

Findings from a rigorously conducted published scientific study by the investigator or other groups, can be proposed under RFA BX-20-013: *BLRD Merit Review Award for Validation of Studies of Importance to Veteran Health*. However, the finding should have a clear translational implication to the Veterans health care, and the validation of the finding should de-risk the future clinical development. Additionally, please check with VA Technology Transfer Program to determine whether the proposed study will result in an invention where VA will have an intellectual property right.

If published data exists in one animal model about a novel target, can we propose to validate this novel target in another animal model of the same disease? And in human iPSC cell culture model?

Yes, if the validation study will de-risk future clinical development and if the target has the potential to address an important health care concerns of our Veterans.