April 12, 2022

ImmunoVec, Inc.
3225 Wilshire, Suite 752
Los Angeles, CA 90010

Re: Application Review Subcommittee meeting for CIRM CLIN1-13315

Dear Independent Citizens Oversight Committee,

We thank CIRM and the Grants Working Group for their favorable reviews and recommendation for funding of our proposal, CLIN1-13315, Hematopoietic Stem Cell Gene Therapy for X-linked Chronic Granulomatous Disease (XCGD).

The reviewers were extremely positive noting:
"*The project is well-planned and well-designed*"
"*This is an excellent team*"
"*project plan is supported by the data available from their extensive pre-clinical studies*"

We also want to thank the reviewers for supporting the goal of ImmunoVec, which is to apply our bioinformatic pipeline to greatly increase the number genetic of disorders that can be treated:
"*The impact of this project will likely be in the proof of principle for other uses of the applicants' bioinformatics-guided approach*"

The overall Tier 1 score from the Grants Working Group indicated the proposal shows "*exceptional merit and warrants funding*".

The reviews contained essentially no criticisms of the existing data or proposed studies, however, some questions arose regarding the diversity equity and inclusion criteria as well as patient outreach, which we would like the opportunity to address here.

PIDTC facilitates access to diverse patient populations:

ImmunoVec co-founder Dr. Donald Kohn has played an integral role for the Primary Immune Deficiency Treatment Consortium (PIDTC). The PIDTC has 47 centers across the United States and Canada that specialize in the care of complex patients with primary immune deficiencies. Centers are in major cities across the US including: Los Angeles, CA, Birmingham, AL, New Orleans, LA, New York, NY, Cincinnati, OH, Memphis, TN and Atlanta, GA. Many of these cities contain very diverse populations, giving patients in these areas access to the specialist physicians trained to diagnose and treat rare immune disorders such as XCGD. The PIDTC also connects patients with advocacy groups (including the CGD Association of America), who function to connect patients of all socioeconomic backgrounds to centers providing treatment (such as UCLA). The PIDTC has committed their extensive resources and clinical databases to assist ImmunoVec in recruiting a diverse patient population (see PIDTC letter of support).

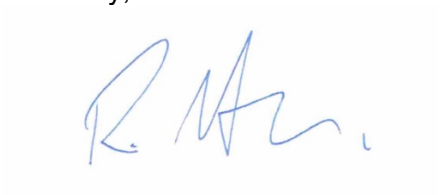
ImmunoVec's commitment to all families:
Participating in this clinical trial may involve extensive travel, and for treatment of pediatric patients, entire families may need to relocate for months. ImmunoVec will cover all clinical trial related costs for patients and their families, including all medical bills, airfare and family housing for the entire hospital admission, which should extend access to patients in need regardless of their financial situation (proposal page 18). As the trial opens up to adolescents and pediatric patients, we fully understand that we will likely have to support more family members and this financial support will be provided regardless of socioeconomic status throughout the treatment process.

Patient advocacy groups:
Highlighting and expanding upon information on page 17 of the application: At ImmunoVec, we have continued to build on existing relationships between *"the Primary Immune Deficiency Treatment Consortium (PIDTC), Immune Deficiency Foundation, Jeffrey Modell Foundation, and Clinical Immunology Society and have also established relationships with UK CGD Society and CGD Association of America. ImmunoVec plans to work with these organizations and patient advocacy groups to advocate for future newborn screening for X-CGD; this will benefit patients traditionally underserved in healthcare to receive an early definitive diagnosis and promote a better outcome for these patients who may otherwise suffer from delayed diagnosis"*. The best campaigners for the patients are the advocacy groups that represent them, we will work closely with the advocacy groups to provide them with trial data and financial support to push for newborn screening of XCGD. We truly feel newborn screening is the only way to fully open up diagnosis to all patients regardless of socioeconomic status.

UCLA's and NIH's reputation to attract patients for rare diseases:
We have deliberately chosen UCLA and NIH to initiate our trials as both centers have an international reputation as referral sites for complex patients with rare diseases. In a recent trial for adenosine deaminase-deficient severe-combined immune deficiency at UCLA, families and physicians have reached out from around the world (including Lebanon and Namibia) to request access to the trial. NIH has a similar track record of international excellence and expertise in the treatment of X-CGD (see support letter from NIH collaborator Dr. Harry Malech) This international renown is key in helping us in our goal to provide access for all patients, regardless of ethnicity, nationality or socioeconomic background.

In conclusion, we sincerely appreciate the GWG's positive feedback on our proposal. ImmunoVec is committed to developing life-changing gene therapies for this fatal pediatric disease and we hope the ICOC will support this project to directly addresses a clear unmet medical need.

Sincerely,



Roger Hollis, PhD
Chief Operating Officer
ImmunoVec