Midpoint Assessment of Outcomes in the ECHO Program Office 2020–2024 Strategic Plan

Completed August 2022

Executive Summary

The purpose of this 2022 assessment is to estimate the extent to which the Environmental influences on Child Health Outcomes (ECHO) program is achieving the outcomes described in the ECHO Program Office 2020-2024 Strategic Plan. This analysis focuses on indicators of the potential impact of ECHO research; the extensiveness of ECHO's data and biospecimen resources; the clinical trial capacity of the ECHO IDeA States Pediatric Clinical Trials Network (ISPCTN); and the progression of ECHO's developing pediatric research workforce.

To help ECHO estimate the potential impact of its research products, the Program Office examined bibliometric indicators: relative citation ratio, journal impact factor, and whether an editorial or commentary cites an ECHO publication. The results suggest that ECHO's multi-award collaborations show promise for higher impact compared to its single-award publications.

To assess the extensiveness of ECHO's data and biospecimen resources, the Program Office pulled related metadata from the ECHO Data Analysis Center and compared them with general expectations projected early in the program. The results indicate that the ECHO Cohort Data Platform and Biorepository are substantial in size but are behind schedule in relation to early expectations.

To estimate ISPCTN clinical trial capacity, the ECHO Data Coordination and Operations Center for ISPCTN provided the Program Office with information on ISPCTN's four completed studies, and one ongoing study. The results from this small sample show that ISPCTN demonstrates capacity to enroll and retain participants in its clinical trials.

To examine the progression of its developing pediatric research workforce, the Program Office analyzed new grant funding and publication records of ECHO's Opportunities and Infrastructure Fund, the first cycle of its Diversity Supplements, and ISCPTN junior investigators. The results suggest that after gaining several years of experience, ECHO's developing scientists demonstrate a strong record of attracting funding and publishing their research.

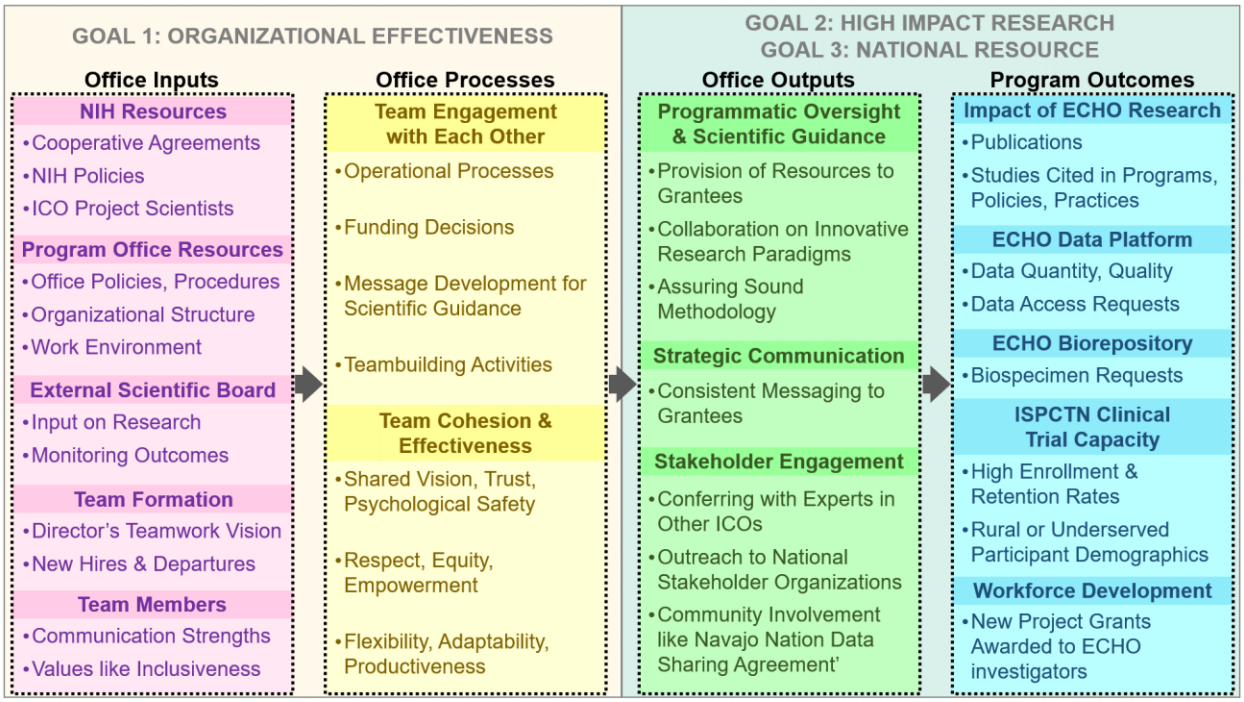
Together, these findings demonstrate ECHO's noteworthy implementation of its strategic plan and accomplishment of several key outcomes. The ECHO Program also demonstrates room for improving the size of the ECHO Data Platform and Biorepository, as well as further development and completion of ISPCTN clinical trials. The appendix of this assessment reports an analysis of team functioning among Program Office staff, showing a higher relative distribution of scores from 2020 to 2021.

Background and Purpose

In 2020, the ECHO Program Office implemented a strategic plan to guide its work through 2024. The plan clarifies the Program Office's mission, strategic goals, and key measures of success for the desired outcomes of the ECHO Program, which includes two major components: a) observational research in the ECHO Cohort and b) intervention research in ISPCTN. ECHO research focuses on five key pediatric outcomes that have a high public health impact: 1) pre-, peri-, and postnatal outcomes, 2) upper and lower airway outcomes, 3) obesity and its consequences, 4) neurodevelopment, and 5) positive health.

The mission of the ECHO Program Office is to promote scientific vision and mobilize nationwide capacity to catalyze observational and intervention research that will enhance the health of children for generations to come. To achieve its mission, the ECHO Program Office set three strategic goals, one corresponding to its organization and two aligning with its desired program outcomes (Figure 1). The three strategic goals are to a) Enhance Program Office Organizational Effectiveness, b) Enable High-Impact Research, and c) Facilitate Establishment of ECHO as a National Resource.

Figure 1. Team-Based Model from the ECHO Program Office Strategic Plan



The ECHO Program Office uses this team-based model to guide implementation and evaluation of its strategic plan. The entries under Office Inputs are examples of assets and resources. Entries under Office Processes are examples of internal activities, as well as indicators of cohesion and effectiveness. Entries under Office Outputs are examples of outward-facing services provided to grantees and various stakeholder groups. Entries under Program Outcomes are example indicators of success for ECHO.

The purpose of this 2022 assessment is to estimate the extent to which ECHO is achieving the desired outcomes listed in the ECHO Program Office 2020–2024 Strategic Plan. As such, this assessment asks one question related to each of the four desired program outcomes:

1. To what extent does ECHO research have potential for high impact?
2. How extensive is the ECHO Cohort central data platform and biorepository?
3. To what extent has ISPCTN built clinical trial capacity?
4. How well has ECHO developed the pediatric research workforce?

In the following sections, this report highlights the rationale, methodology, findings, and limitations for each question. The appendix of this report includes a two-year analysis of the Program Office's goal to Enhance Program Office Organizational Effectiveness.

Analysis Questions

Question 1: To what extent does ECHO research have potential for high impact?

Rationale and Methodology

The ECHO Program Office characterizes impact as measurable enhancements in child health in the five ECHO health outcome domains. According to the vision for the program, ECHO contributes to these enhancements by providing research results that inform programs, policies, or practices. Such impact can take decades to emerge, so the Program Office has selected bibliometric indicators to help ECHO estimate the potential impact of its research products in the short- and medium-term. These indicators include relative citation ratio (RCR), journal impact factor (JIF), and whether an editorial or commentary cites an ECHO publication.

To assess the indicators, an analyst batched PubMed IDs by category of paper:

1. ECHO Cohort papers involving multiple-cohort awards
2. Single-award papers for centers, cohorts, or cores ($^{\text{Cohort}}$ Single Award)
3. ISPCTN papers involving multiple ISPCTN awards
4. Single-award papers for ISPCTN clinical sites or the Data Coordinating and Operations Center ($^{\text{ISPCTN}}$ Single Award)

The analyst searched each batch of PubMed IDs in various databases:

1. NIH's iCite to pull RCR data
2. NIH's SPIRES to pull JIF data
3. NIH's iSearch Publications to document any citation of each PubMed ID in editorials or commentaries

Findings

ECHO's multi-award collaborations show promise for higher impact than its single-award publications do (Table I and Figure 2).

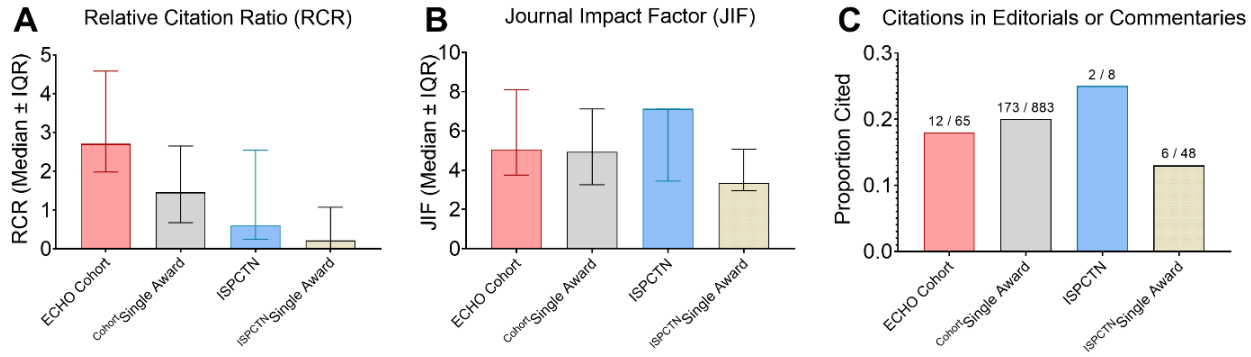
i. **Bibliometric indicators of potential impact of ECHO Cohort research.** As of August 2022, the ECHO Cohort Consortium has published nearly 70 ECHO Cohort manuscripts and just over 900 single-award manuscripts The median RCR for ECHO Cohort publications is ~2.8, corresponding to the ~85th percentile of NIH-funded papers (Figure 1, panel A). The median RCR for single-award papers is ~1.5, equivalent to the ~65th percentile of papers. Journals with similar impact factors publish articles in both the ECHO Cohort and single-award categories (~5 median JIF, Panel B). Papers in both categories receive citations in editorial and commentaries in similar proportions (~0.2 median, Panel C).

ii. **Bibliometric indicators of potential impact of ECHO ISPCTN research.** ISPCTN publications total 8 for ISPCTN multi-award papers and 53 for single-award papers. Panel A shows that the median RCR is ~0.6 for multi-award papers and ~0.2 for single-award papers. Panel B shows that the median impact factor was just over 7 for ISPCTN multi-award papers and ~3 for single-award papers. Panel C shows that editorials or commentaries cited 25% of ISPCTN multi-award papers compared to 13% for single-award papers.

Table I. Publication totals and data availability in iCite and SPIRES

	Total Publications	Number of publications with RCR data in iCite	Number of publications with JIF data in SPIRES
ECHO Cohort	66	25	42
Single Award (cohort, core, center)	910	585	571
ECHO ISPCTN	8	5	4
Single Award (ISPCTN)	53	33	21

Figure 2. Bibliometric Indicators of ECHO Research



Limitations

One limitation is that it generally takes one year after publication for iCite to register an interim RCR for an article. iCite will assign an interim value earlier to papers that receive substantial citation rates during the first year after publication. Therefore, the number of papers eligible for RCR is lower than the number published. Another limitation is that SPIRES does not contain impact factors for all journals listed in the database. Table I highlights the number of data points missing from the analysis presented in Figure 1. A third limitation is that iSearch does not currently categorize links between an article and an accompanying editorial or commentary. The current approach only identifies when an editorial or commentary cites an ECHO paper, which returns more articles than those truly accompanied by an editorial or commentary. A fourth limitation is that ISPCTN has not published enough papers yet for precise comparisons.

Question 2: How extensive is the ECHO Cohort central data platform and biorepository?

Rationale and Methodology

Enabling high-impact research and establishing ECHO as a national resource involve populating a central ECHO Cohort Data Platform and a central ECHO Cohort Biorepository. In addition to examining the sizes of the platform and repository, the analysis below examines the number of participants in active follow-up, which affects the rate at which the data platform and biorepository can expand.

To assess the size of the platform and repository, an analyst pulled related metadata from the ECHO Data Analysis Center's administrative reports and ECHO PlaTIPUS (ECHO Cohort Platform: Inform and Provide User Search). For context, the analysis compares the sizes of the platform and repository with some general expectations from early in the ECHO program.

Table II. Size of ECHO Cohort Data Platform and Biorepository

ECHO Cohort Data Platform
• 115,727[1] participants, including 62,437[2] children, have contributed extant or new data including over 565,000[3] newly collected data elements from 26,784[4] mother-child dyads. • ECHO expected over 1 million[5] data elements.
ECHO Cohort Biorepository
• 29,924[6] participants, including 15,006[7] children, contributed 60,856[8] biospecimens including 27,749[9] biospecimens from children. • ECHO had expected over 350,000[10] unique biospecimens.
Active Participant Follow-Up
• 31,541[11] child participants have enrolled in ECHO for active follow-up • ECHO had expected ~45,000[12] child participants enrolled for active follow-up.

[1] Overall participants contributing extant and new data from form D5b, tab 2, dated 08/15/2022

[2] Data reported in PlatIPUS (August 2022)

[3,4,5] From report D11 (August 2022); note essential data elements as described in the ECHO-wide Cohort Protocol v1.0 from 06/21/18; there are more data on the platform but not clear how to quantify

[6]Participant IDs from report C1 dated 07/17/2022

[7] From report C3c dated 07/17/2022

[8] Number of non-DNA samples reported in Goals, Objectives, Indicators, and Targets Dashboard for August 2022 plus saliva samples listed in report C1 (July 2022; note: no file saved for August)

[9] Sum of Overall columns in report C3c dated 07/17/2022; note: no file saved for August

[10] From analysis showing that ECHO anticipated and budgeted for kits for collection of 350,000 biospecimens from participants in active follow up from 2017–2023

[11,12] Report A3 dated 07/18/2022

Findings

The ECHO Cohort Data Platform and ECHO Cohort Biorepository are substantial in size, but each has grown more slowly than predicted early in the program (Table II).

i. **Data Platform.** Overall, as of August 2022, more than 115,000 participants, including over 62,000 children, have contributed data according to the ECHO-wide Cohort Protocol. Of these participants, approximately 26,000 Level 2 mother-child dyads with at least 12 months in a life stage have provided data on over 565,000 essential data elements to the data platform. According to the Data Analysis Center's administrative report D11 in August 2022, ECHO had expected collection of approximately 1.2 million essential data elements from these Level 2 participants on the platform by August 2022.

ii. **Biorepository.** The ECHO Cohort Biorepository includes over 60,000 biospecimen samples from nearly 30,000 participants, including over 27,000 biospecimen samples from 15,000 children. Early in the program, ECHO budgeted for collection of over 350,000 biospecimen samples by the summer of 2023.

iii. **Participants in Active Follow-Up.** As of July 2022, the ECHO Cohort comprises nearly 31,500 child participants in active follow-up. Early in the program, ECHO expected to enroll ~45,000 children in active follow-up by the summer of 2023.

Limitations

One caveat in this analysis is a lack of clear parameters to characterize the size of the ECHO Cohort Data Platform. For example, there are variations in how ECHO counts participants, e.g., through various levels of participation, as well as through implicit and explicit registrations. The unit of "data element" includes multiple intra-unit classifications, e.g., essential and recommended measures, as well as extant and new data. ECHO also allowed a lot of flexibility to cohorts regarding data collection, visit schedule, and choreography. The COVID-19 pandemic, extended closures at many ECHO clinical sites, and disruptions in the lives of participants caused additional challenges.

Question 3: To what extent has ISPCTN built clinical trial capacity?

Rationale and Methodology

A major focus of ECHO's goal to enable high-impact research is to build ISPCTN capacity to provide children in rural or underserved communities of IDeA States with access to cutting-edge multi-center clinical trials. To estimate ISPCTN clinical trial capacity, the ECHO Data Coordination and Operations Center for ISPCTN provided the ECHO Program Office with information on ISPCTN's completed studies, as well as one active study. The information includes the overall design of each study, e.g., clinical trial, observational, or qualitative. For

each study, the center provided the total number of sites contributing, enrollment and retention numbers, and number of publications resulting.

Findings

In a small sample, as of August 2022, ISPCTN demonstrates capacity to enroll and retain clinical trial participants (Table III).

i. **ACT NOW Current Experience (ACT NOW CE).** This observational, cross-sectional study involved medical record abstraction across 26 study sites. It did not require enrollment and follow-up efforts. The study team has produced four publications associated with ACT NOW CE.

ii. **VDORA1.** This clinical trial involved 17 study sites that enrolled 114 participants (expected enrollment of 113). Of the 114 participants randomized, the study team retained 104 through study completion. The study team has one publication associated with VDORA1.

iii. **iAmHealthy.** This clinical trial involved four study sites that enrolled 104 participants (expected enrollment of 112). Of the 104 participants randomized, the study team retained 95 through study completion. The study team has one publication associated with iAmHealthy.

iv. **MoVeUP Qualitative.** This focus group involved four study sites that engaged 36 participants of the 69 scheduled for participation. The study team has no publications associated with MoVeUP Qualitative.

v. **ACT NOW Eating, Sleeping, Consoling (ACT NOW ESC**)**.** This ongoing clinical trial in active follow-up involves 26 study sites with an enrollment of 675 participants (expected enrollment of 1034).

Table III. Indicators of ISPCTN Clinical Trial Capacity

Study Title (Category)	Number of Sites	Enrollment[a] (Proportion)	Retention[b] (Proportion)	Number of Associated Publications
ACT NOW CE (Observational)	26	1808	N/A	4
VDORA1 (Clinical Trial)	17	114 / 113 (1.01)	104 / 114 (0.91)	1
iAmHealthy (Clinical Trial)	4	104 / 112 (0.93)	95 / 104 (0.91)	1
MoVeUP (Qualitative)	4	36 / 69 (0.52)	N/A	0
ACT NOW ESC (Clinical Trial)	26	675 / 1034 (0.65)	In Follow-Up	0

[a]Number of participants enrolled / expected enrollment

[b]Number of participants who completed study / number of participants randomized

N/A—Not available

Limitations

The small number of completed ISPCTN studies presents a challenge to this analysis. Discerning patterns of clinical trial capacity would require a larger sample of completed studies. Lack of a comparison group or frame of reference makes it difficult to put this analysis in context. In the absence of other networks to use as comparisons, tracking these indicators for ISPCTN over time can provide an internal standard for assessing approaches to improvement.

Question 4: How well has ECHO developed the pediatric research workforce?

Rationale and Methodology

The ECHO Program Office committed to enabling high-impact research across the program by, among other actions, developing the pediatric research workforce. With the added context of building clinical trial capacity in ISPCTN addressed in the previous question, this analysis focuses on two key attributes of maturing investigators: a) the ability to attract research funding and b) a record of primary authorship of research publications.

This analysis starts with a look at ISPCTN principal investigators and then examines ECHO investigators in earlier career stages including investigators from the first three cycles of ECHO's Opportunities and Infrastructure Fund (OIF), the first cycle of ECHO's Diversity Supplements, and ISCPTN junior investigators.

An analyst compiled lists of names for investigators within each category or cycle. The analyst used NIH's iSearch Grants database to retrieve grants listing the investigators above as Contact Principal Investigator or Other Principal Investigator, not counting ECHO parent awards. The analyst pulled data from iSearch Publications to retrieve a) publications listing any of the ISPCTN principal investigators as the first or senior author; or b) any of the OIF, Diversity Supplement, or ISPCTN Junior investigators as first author. The method of searching for first- or last-author publications among ISPCTN principal investigators opened the opportunity for double reporting some publications. However, reviewing the combined list of publications identified only two overlapping papers, which the analyst subtracted from the final total.

Of the 36 ISPCTN investigators included, the analysis start date for 32 investigators is 2016 based on the assumption they joined ISPCTN when the program launched. The analysis start date for the remaining four investigators is 2020 based on the assumption they joined ISPCTN as Principal Investigator at the start of Cycle 2.

For comparison, the analyst also pulled the same grant and publication data from applicants to OIF cycles 1–3 and Diversity Supplement cycle 1 whom ECHO did not select for funding.

Findings

ECHO's developing scientists are attracting funding and publishing their research after gaining several years of experience. Together as a group, ISPCTN investigators earned 17 new project grants during the analysis period, a rate of approximately one new project grant for every two

investigators (Table IV). Over the same analysis period, the 36 ISPCTN investigators accounted for 180 first- or last-author publications, i.e., five publications per investigator on average.

Table IV. Grant and Publication Information for Developing ECHO Investigators

	Investigator Count	Analysis Start	Number of New Project Grants (per investigator)	Number of First- or Last-Author Publications (per investigator)
ISPCTN Investigators	36	2016[a]	17 (0.5)	180 (5.0)

[a]2016 start for 32 investigators, 2020 for 4

i. **Opportunities and Infrastructure Fund (Table V).** Overall, OIF investigators over the first three cycles have more project grants and more first-author publications than do applicants whom ECHO did not select for funding. The differences in number of new project grants per investigator are larger for Cycle 1 (0.9 vs. 0.4) and Cycle 3 (0.3 vs. 0.1) than in Cycle 2 (0.9 vs 0.8). Similarly, the differences in first-author publications per investigator are larger for Cycle 1 (5.3 vs. 2.9) and Cycle 3 (4.9 vs. 2.8) than in Cycle 2 (4.0 vs. 3.5).

ii. **Diversity Supplements (Table V).** The eight Diversity Supplement awardees, as well as the three investigators whom ECHO did not select for funding with these supplements, have not received any new project grants since 2020. While the total number of first-author publications is higher for Diversity Supplement investigators than those whom ECHO did not select for funding (6 vs. 5), the per investigator average is lower for Diversity Supplement investigators (0.8 vs. 1.7).

iii. **ISPCTN Junior Investigators (Table V).** NIH has awarded five new project grants among the 35 ISPCTN Junior Investigators (0.14 per investigator) since 2020. The same group has produced 29 first-author publications during the same time.

Table V. Grant and Publication Information for Developing ECHO Investigators

	Investigator Count	Analysis Start	Number of New Project Grants (per investigator)	Number of First Author Publications (per investigator)
OIF Cycle 1	10	2018	9 (0.9)	53 (5.3)
Not Selected for OIF Cycle 1	12	2018	5 (0.4)	35 (2.9)
OIF Cycle 2	11	2019	10 (0.9)	44 (4.0)
Not Selected for OIF Cycle 2	6	2019	5 (0.8)	21 (3.5)
OIF Cycle 3	9	2020	3 (0.3)	44 (4.9)
Not Selected for OIF Cycle 3	11	2020	1 (0.1)	31 (2.8)
Diversity Supplements Cycle 1	8	2020	0[a]	6 (0.8)
Not Selected for Diversity Supplements Cycle 1	3	2020	0[a]	5 (1.7)
ISPCTN Junior Investigators	35	2020	5 (0.14)	29 (0.8)

[a]Diversity Supplement Scholars and applicants are at earlier career stages than are OIF investigators.

Limitations

Uncertainty about the precision of analysis start dates for principal investigators in terms of investigator transitions during Cycle 1 and Cycle 2 of ISPCTN, and uncertainty in whom to include in the analysis as ISPCTN Junior Investigators, limits this analysis. For the analyses starting in 2020, more time may need to pass before ECHO can do meaningful assessment of progress for OIF and Diversity Supplement awardees, and for ISPCTN Junior Investigators.

Summary and Conclusion

This assessment of ECHO program outcomes finds, most significantly:

a) ECHO's multi-award collaborations show promise for higher impact than single-award publications do.
b) The ECHO Cohort Data Platform and Biorepository have reached substantial size but have not yet met early expectations.
c) ISPCTN demonstrates capacity to enroll and retain clinical trial participants, in a small sample of studies.
d) ECHO's developing scientists succeed in attracting funding and publishing their research after gaining several years of experience.

Taken together, these findings demonstrate that the ECHO Program has achieved noteworthy progress toward many of its desired outcomes. This suggests that the Program Office is successfully implementing its strategic plan. The ECHO Program also demonstrates substantial room for improvement in the remaining time under the current Strategic Plan. Specific activities ripe for improvement include expanding the size of the ECHO Data Platform and Biorepository, as well as further development and completion of ISPCTN clinical trials.

Appendix

The strategic goal to Enhance Program Office Organizational Effectiveness addresses how the Program Office works to help the ECHO Program achieve its desired outcomes. These work activities relate to enhancing the Office's organizational structure, operational processes, and interpersonal cohesion.

Rationale and Methodology

The ECHO Program Office uses an integrative approach to understanding the extent of its organizational effectiveness based on the concept of Team Functioning, which comprises the emergence of a) affective qualities like trust and psychological safety, b) group cognition like shared understanding of team roles and collaborative work, and c) behavioral features like team cohesiveness and effectiveness. Higher levels of team functioning should indicate that, collectively, staff capitalize on their available resources and internal processes to influence more effectively achievement of program outcomes.

To assess team functioning, staff complete an annual questionnaire that draws from the Team Effectiveness Questionnaire used by the UK National Health Service's London Leadership Academy, with slight modifications to clarify some terms for use in the ECHO Program Office. The questionnaire has 56 items that staff have mapped to domains of the model shown in Figure 1 like team formation, team members, office resources, team engagement, and team output (effectiveness). Staff completed the questionnaire in 2020 and 2021.

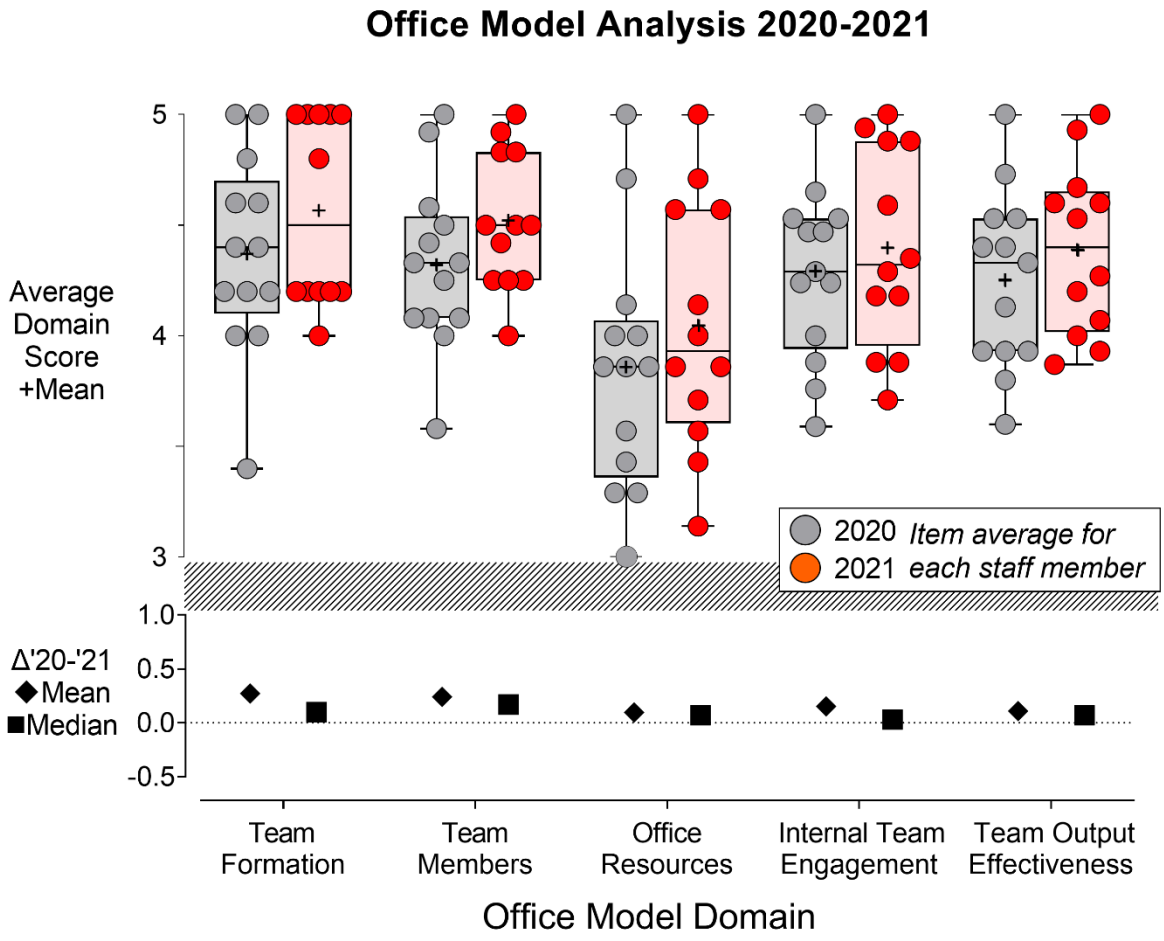
Findings

From 2020 to 2021, ECHO Program Office staff gave higher scores on most questionnaire items, resulting in a higher relative distribution of scores overall (Table AI and Figure A1).

Table AI. Overall Distribution of Office Team Functioning Results

	2020 (N=13)	2021 (N=12)	Δ
Possible Range	5–25	5–25	N/A
Mean	21.1	21.9	+0.9
Min	17.2	19.7	+2.5
Max	25.0	25.0	0.0
Standard Dev	2.1	2.0	-0.1

Overall scores were high. Among domains, staff attributed higher levels of agreement with positive statements about team formation and team members compared to positive statements about office resources, internal team engagement, and team output effectiveness.



Summary

Taken in the context of program outcomes, ECHO Program Office staff report high levels of team functioning, which addresses the goal of enhancing organizational effectiveness. Since staff completed the questionnaire while on full-time telework during the COVID-19 pandemic, higher scores in 2021 compared to 2020 may reflect more time working virtually together—in addition to some staff working together before the pandemic. Also, few staff joined or left the team in 2020 and 2021. These two considerations suggest that staff perhaps experienced substantial bonding. The high values that staff reported for team formation and team members support this conclusion. Staff may have also improved their teleworking skills and competencies for improving their overall work experience.