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## **Research and Statistics : Validity Hierarchy for Study Design and Study Type**

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# Validity Hierarchy for Study Design and Study Type

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## Case Studies

- *The parents of a child in whom you have diagnosed attention-deficit/hyperactivity disorder (ADHD) are concerned about starting the boy on stimulant medicine. They want to know if behavioral interventions can be as effective as stimulant medicine.*
- *At a 1-year health supervision visit, a first-time mother expresses concern about giving her child the measles, mumps, and rubella (MMR) vaccine. She read several websites that recommended not allowing a child to receive the vaccine because of possible links to autism.*

## Introduction

Identifying and implementing effective, evidenced-based care is considered best practice in pediatrics. An evidence-based clinician reviews the current literature to understand the evidence before addressing the concerns of parents such as those in the case studies, but it is important to determine what counts as good evidence. Engaging in evidence-based practice requires the clinician to interpret the evidence from research studies. Research study design and type are important considerations when determining if the conclusions of the study are valid and to provide sufficient evidence to guide clinical decisions. Both study design and type influence the validity of the research study.

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## Validity Hierarchy

Validity hierarchy is based on the internal validity of the study design and type (Table). Internal validity reflects the accuracy of the study's conclusions and is needed to determine causal relations among variables; external validity reflects how well a study represents the "real world." Different study designs (eg, experimental versus nonexperimental) and study types (eg, cohort, cross-sectional, and case studies) offer varying advantages and disadvantages for answering research questions. Feasibility, cost, and ethical considerations also influence which study designs and types can be used to answer the research question.

## Experimental Studies

Randomized, controlled trials (RCTs) are considered the gold standard of research designs. RCTs have high internal validity because several aspects of study design are controlled. For example, study participants are assigned randomly to treatment or comparison groups to reduce the chance of differences between the two groups at baseline. Thus, RCTs should allow a researcher to conclude that the intervention causes or is responsible for different outcomes between the groups, rather than other baseline differences (eg, the use of stimulant medications was the reason for the difference between two groups of children who have ADHD). In other study designs, these baseline differences may result in confounding, which occurs when another variable may explain the different outcomes between groups. For example, the

Table. **Validity Hierarchy**

 Internal Validity	Study Design	Strengths	Weaknesses
	Randomized controlled trials	<ul style="list-style-type: none"> <li>• High internal validity</li> <li>• Reduced risk of confounding variables</li> </ul>	<ul style="list-style-type: none"> <li>• Reduced external validity</li> <li>• Expensive, time-consuming</li> </ul>
	Cohort studies	<ul style="list-style-type: none"> <li>• Useful for sequential events</li> <li>• Can study multiple outcomes</li> <li>• <i>Retrospective</i>: less expensive</li> </ul>	<ul style="list-style-type: none"> <li>• Requires large sample size</li> <li>• Risk of confounding variables</li> <li>• Difficult to study rare outcomes</li> <li>• <i>Prospective</i>: Expensive</li> </ul>
	Case-control studies	<ul style="list-style-type: none"> <li>• Useful for rare outcomes</li> <li>• Can study several exposures</li> <li>• Inexpensive</li> </ul>	<ul style="list-style-type: none"> <li>• Risk of confounding variables</li> </ul>
	Cross-sectional studies	<ul style="list-style-type: none"> <li>• Can study multiple outcomes and exposures</li> </ul>	<ul style="list-style-type: none"> <li>• Cannot infer causality</li> <li>• Risk of confounding variables</li> <li>• Less useful for rare exposures or outcomes</li> </ul>
	Case studies	<ul style="list-style-type: none"> <li>• Useful for rare outcomes</li> <li>• Convenient, inexpensive</li> </ul>	<ul style="list-style-type: none"> <li>• Risk of confounding variables</li> <li>• Lack of a comparison group</li> <li>• Cannot infer causality</li> </ul>

Adapted from Ho, et al. *Circulation*. 2008;118:1675–1684.

differences in symptom control between children who received stimulant medication and those who did not may not be a result of the exposure to the stimulant medication, but rather due to the children who received the stimulant medication being older or having fewer ADHD symptoms at baseline. A major strength of RCTs is the reduced influence of confounding variables by making the groups equivalent at baseline.

In addition, RCTs often use “blinding” of both the researcher and participants to eliminate bias in interpreting the outcomes. However, RCTs can be expensive and time-consuming to conduct. Moreover, some research questions do not lend themselves to an RCT design. For example, consider the challenges in designing an RCT to investigate the potential links between the MMR vaccine and autism. Given the known risks of infection with measles, mumps, and rubella, it would be unethical to withhold the MMR vaccine from children as part of randomization.

### Nonexperimental Studies

In cohort studies, a sample of people (or cohort) can be followed over time to evaluate risks for a future outcome (prospective cohort study) or traced back to investigate potential historic risks for a current outcome or diagnosis (retrospective cohort study). The major strength of the cohort design is that researchers can examine the risk of a particular exposure (eg, receiving MMR vaccine) for a certain outcome (eg, development of autism). However, cohort studies require a large sample size, and it often is not possible to control for exposure to other variables that could influence the outcomes (ie, confounding variables).

An approach that can be used with smaller sample sizes and rare outcomes is the case-control design, in which the researcher can compare cases (eg, children who have autism) with controls (eg, children who do not have autism) regarding exposure to a risk factor (eg, MMR vaccination). Case-control studies have the benefit of controlling for differences between the cases and controls be-

cause the researcher can match cases and controls according to age, sex, or other relevant factors. However, the case-control design has lower internal validity because the researcher frequently must rely on recall of historical facts (eg, maternal recall of first emergence of autistic symptoms and timing of MMR vaccine).

Case studies also can be used to explore rare events and to identify clinical questions that need more study. It is important to remember that a case study only provides evidence for the one or few cases studied.

Another nonexperimental design is the cross-sectional study, in which the researcher collects data about factors of interest at one point in time. The convenience of cross-sectional studies makes them more practical to implement than designs higher on the validity hierarchy. Cross-sectional studies allow a researcher to examine potential associations between two measured factors at one point in time. Because the factors are measured at the same time, one limitation of this design is

that it cannot provide evidence of causality.

Nonexperimental designs are vulnerable to the effects of confounding variables. Thus, the inability to infer causality is a prominent disadvantage. However, as noted previously, researchers often must rely on nonexperimental designs due to ethical and logistical concerns. Such studies can yield important and beneficial information and frequently lay the foundation for later RCTs.

### Considering the Case Studies

Considering the case studies in light of the validity hierarchy can aid in providing answers to the parents. Regarding ADHD treatments, a large-scale, multisite RCT showed that stimulants alone or in combination with behavioral interventions are more effective than behavioral interventions alone or usual community care. This RCT has high internal va-

lidity and, therefore, provides robust evidence to make an appropriate clinical decision regarding ADHD treatments. In contrast, the hypothesized causal link between the MMR vaccine and autism has not been evaluated by using an RCT design. However, many retrospective and prospective cohort and case-control studies repeatedly have failed to link the MMR vaccine to the development of autism. Thus, the available evidence that refutes the hypothesized association between MMR vaccines and autism can guide clinical decisions.

### Conclusion

The validity hierarchy provides a guide to interpreting the level of evidence that a study can provide for a particular research question. RCTs may not address all clinical questions due to risk of harm in the treatment or inability to randomize patients

ethically to the intervention (eg, withholding vaccines). When RCTs are not available, other studies that have sufficiently large samples of participants and consistent results among multiple studies can provide compelling evidence for making good clinical decisions.

### Suggested Reading

- American Academy of Pediatrics. *Vaccine Studies: Examine the Evidence*. Accessed April 2009 at: [www.cisimmunize.org/Vaccine%20Studies.pdf](http://www.cisimmunize.org/Vaccine%20Studies.pdf)
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