

## Key Findings

- **Clinicians overwhelmingly cited multiple needs for new or improved medical devices for diagnosing and treating rare diseases**



461 unique rare diseases were cited with 917 specifying unmet device needs  
91% believed a new or improved device is needed  
64% were dissatisfied with existing diagnostic and/or therapeutic devices

- **There is a critical need for entirely new devices rather than modifying or repurposing devices, which are often inadequate**



77% cited a need for an entirely new diagnostic and/or therapeutic device  
23% cited a need for only modified or repurposed diagnostic and/or therapeutic devices

- **Existing devices have several limitations in diagnosing or treating rare diseases**



79% reported diagnostic devices for genetic disorders as an unmet need  
37% currently repurpose an FDA-approved therapeutic device

- **Several impediments to developing new devices for rare diseases were mentioned**



74% saw the lack of profitability to industry as a large impediment  
67% saw the cost of development as a large impediment

- **The Humanitarian Device Exemption (HDE) provides a helpful pathway for bringing devices to market, but there are obstacles to its use.**



*Top challenges cited by the 51% of respondents reporting familiarity with HUD/HDEs include the following:*

52% said reimbursement  
50% reported gaining access to HDE devices  
46% indicated institutional review board constraints

- **While there are unique pediatric challenges, respondents with pediatric experience reported high levels of dissatisfaction similar to those without pediatric experience**



33% of clinicians had a pediatric focus  
66% believed there is a pediatric need for implants that grow along with the child  
44% confirmed intrathecal ports for drug delivery confirmed as a pediatric need