Research priorities for endometriosis differ among patients, clinicians, and researchers

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Title: Research priorities for endometriosis differ among patients, clinicians, and researchers

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Objective:
Endometriosis—the implantation of endometrial-like tissue outside of the uterus—burdens an estimated 6-10% of women worldwide and is associated with chronic pelvic pain and infertility.\(^1,2\) Currently, the standard method of diagnosis is surgery, which creates barriers to care and delays in diagnosis.\(^3\) As a result, many questions fundamental to our understanding of endometriosis remain unanswered. The objective of this work is to identify which research questions are most meaningful to those personally affected by the disease, as compared to those treating and studying it.

Study Design:
In 2016, a James Lind Alliance (JLA) Endometriosis Priority Setting Partnership was formed to identify endometriosis-related questions of the highest priority in the United Kingdom.\(^4\) Research questions were gathered by online survey using well-established JLA methodology\(^5\) complemented by systematic reviews and clinical guidelines. As surveys were completed anonymously and data were deidentified, this research was exempt from Institutional Review Board approval. Ultimately, 72 questions were ranked by 1,418 patients, family and friends, and healthcare practitioners, to generate a final list of ten research priorities.\(^4\) Rankings from 339 international respondents were excluded in the initial publication, and included here.

Subsequently, at the 13\(^{th}\) World Congress of Endometriosis in 2017, 298 international healthcare practitioners and scientists ranked the top 72 questions identified previously, via survey.\(^4\) The purpose of this follow-up study was to compare the priorities for respondents personally affected by endometriosis (patients and close family), with those of clinicians and scientists focusing on endometriosis.
Questions were arranged into ten subject areas (Table). Respondents’ rankings of subject areas were analyzed according to personal diagnosis of (and/or close family member with) endometriosis, employment as healthcare provider or scientist, and gender. Multivariable log-binomial regression modeled the relative risk and 95% confidence interval of prioritization of each subject area, comparing responses from patients/families (referent) to those of healthcare providers/scientists, adjusting for age (< or >45 years) and gender; respondents fitting into both groups were excluded from these comparison analyses. In secondary analyses, we compared male respondents to female (referent) respondents adjusting for respondent type (Patient/family member, healthcare provider/researcher, or both) and age.

**Results:**

Overall (n=2,055), a majority of respondents prioritized research questions about endometriosis treatment (84.2%). As compared to patients/family members (n=1,575), healthcare providers/scientists (n=245) were more likely to prioritize research questions about cause/pathology or risk factors for endometriosis, diagnosis and screening, treatment, and fertility (Table). Patients and family members were more likely to prioritize questions about education/awareness, emotional impact, and comorbid conditions. Priorities of respondents who were both patients/families and providers/scientists (n=235) were nearly identical to responses of patients/families (not shown). Male respondents were more likely to prioritize cause/pathology or risk factors, cure, and fertility, while female respondents prioritized education/awareness, emotional impact, and alternative therapies, even after adjustment for responder category (patients/families vs. providers/scientists).

**Conclusion:**
While clinicians and scientists largely dictate research objectives, this study demonstrates that the priorities of patients and their families differ in a number of key areas, including education and emotional effects of endometriosis. In the era of patient-centered care, this confirms that patients’ questions and concerns must be incorporated into research directions, and currently, certain needs and interests of endometriosis patients may not be adequately recognized.

Acknowledgements

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References

<table>
<thead>
<tr>
<th>Group (n)</th>
<th>1 Cause, Risk, Pathology</th>
<th>2 Cure</th>
<th>3 Education, Awareness</th>
<th>4 Diagnosis, Screening</th>
<th>5 Fertility</th>
<th>6 Emotional Impact</th>
<th>7 Treatments</th>
<th>8 Pain</th>
<th>9 Alternative Treatments</th>
<th>10 Comorbid conditions</th>
</tr>
</thead>
<tbody>
<tr>
<td>All (2,055)</td>
<td>1455 (70.8%)</td>
<td>1289 (62.7%)</td>
<td>1369 (66.6%)</td>
<td>1350 (65.7%)</td>
<td>923 (44.9%)</td>
<td>995 (48.4%)</td>
<td>1730 (84.2%)</td>
<td>1175 (57.2%)</td>
<td>871 (42.4%)</td>
<td>1387 (67.5%)</td>
</tr>
<tr>
<td>Patient and/or family member (1,575) (Referent)</td>
<td>1094 (69.5%)</td>
<td>969 (61.5%)</td>
<td>1119 (71.0%)</td>
<td>997 (63.3%)</td>
<td>683 (43.4%)</td>
<td>812 (51.6%)</td>
<td>1304 (82.8%)</td>
<td>934 (59.3%)</td>
<td>687 (43.6%)</td>
<td>1092 (69.3%)</td>
</tr>
<tr>
<td>Healthcare Provider or Researcher (245)</td>
<td>200 (81.6%)</td>
<td>174 (71.0%)</td>
<td>81 (33.1%)</td>
<td>191 (78.0%)</td>
<td>148 (60.4%)</td>
<td>57 (23.3%)</td>
<td>218 (89.0%)</td>
<td>136 (55.5%)</td>
<td>81 (33.1%)</td>
<td>149 (60.8%)</td>
</tr>
<tr>
<td>Females (1,926) (Referent)</td>
<td>1351 (70.1%)</td>
<td>1184 (61.5%)</td>
<td>1317 (68.4%)</td>
<td>1255 (65.2%)</td>
<td>848 (44.0%)</td>
<td>963 (48.0%)</td>
<td>1613 (83.7%)</td>
<td>1102 (57.2%)</td>
<td>833 (43.3%)</td>
<td>1305 (67.8%)</td>
</tr>
<tr>
<td>Males (245)</td>
<td>100 (82.6%)</td>
<td>100 (82.6%)</td>
<td>46 (38.0%)</td>
<td>89 (73.6%)</td>
<td>72 (59.5%)</td>
<td>29 (24.0%)</td>
<td>109 (57.0%)</td>
<td>69 (37.0%)</td>
<td>37 (24.0%)</td>
<td>76 (37.0%)</td>
</tr>
<tr>
<td>Relative Risk</td>
<td>1.18 (1.10, 1.26)</td>
<td>1.15 (1.06, 1.26)</td>
<td>0.47 (0.39, 0.56)</td>
<td>1.23 (1.14, 1.33)</td>
<td>1.39 (1.24, 1.56)</td>
<td>0.45 (0.36, 0.60)</td>
<td>1.07 (1.02, 1.13)</td>
<td>0.94 (0.83, 1.05)</td>
<td>0.76 (0.63, 0.91)</td>
<td>0.88 (0.79, 0.98)</td>
</tr>
<tr>
<td>Adjusted Relative Risk</td>
<td>1.15 (1.05, 1.26)</td>
<td>1.01 (0.89, 1.16)</td>
<td>0.46 (0.37, 0.59)</td>
<td>1.24 (1.12, 1.36)</td>
<td>1.51 (1.30, 1.76)</td>
<td>0.51 (0.38, 0.69)</td>
<td>1.08 (1.01, 1.15)</td>
<td>0.99 (0.85, 1.15)</td>
<td>0.93 (0.74, 1.17)</td>
<td>0.80 (0.69, 0.92)</td>
</tr>
</tbody>
</table>

1 Respondents who were both patients/family members and clinicians or researchers (n=235) were excluded from this analysis.
2 Log-binomial regression was used to calculate the relative risk of prioritizing each subject area (dependent variable) comparing patient and/or family member with endometriosis (referent) to health care provider or researcher.
3 Risk ratios were adjusted for gender and age (<45 vs. >45 years).
4 Respondents who did not state their gender (n = 8) are excluded from this analysis.
5 Log-binomial regression was used to calculate the relative risk of prioritizing each subject area (dependent variable) comparing males with females (referent).
6 Risk ratios were adjusted for respondent type (Patient/family member, health care provider/researcher, or both) and age (<45 vs. >45 years).