Congenital Cholesteatoma

Classification, Management, and Outcome

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Objectives: To assess whether a classification system for congenital cholesteatoma (CC) can be derived from analysis of a large clinical sample of cases and to assess whether such a classification system is a reliable guide for surgical intervention, reexploration, and hearing outcome.

Design: A retrospective review of clinical and surgical records of 119 patients with CC.

Setting: Four tertiary care children’s hospitals.

Patients: One hundred nineteen children with CC (age range, 2-14 years).

Results: Congenital cholesteatomas in the anterior mesotympanum were treated successfully with exploratory tympanotomy. Congenital cholesteatomas involving the posterior superior quadrant and the attic usually had concurrent involvement of the incus and stapes and often required a canal wall up tympanomastoidectomy and a second look for its control. Congenital cholesteatoma involving the mastoid usually involved all of the ossicles, was inconsistently controlled with canal wall up tymanomastoidectomy, and had a poor prognosis for restoration of conductive hearing loss. The mean±SD age of children with CC was 5.6±2.8 years, while that of children with acquired cholesteatoma was 9.7±3.3 years.

Conclusions: The sequence of spread of CC, involving 3 sites, suggests a natural classification system. The CC usually originates in the anterior superior quadrant, but does not consistently remain there, and may variably occupy the middle ear and mastoid and result in ossicular destruction and conductive hearing loss. The location of CC and the involvement of the ossicles is an accurate predictor of the type of surgery necessary for its control and for the success of hearing restoration.


Thirty-seven years after Derlacki and Clemis1 established our contemporary understanding of congenital cholesteatoma (CC), the disease remains controversial, surgically challenging, and of compelling interest to otolaryngologists. Various techniques are successful in the surgical management of early lesions, but surgery for advanced disease is often undermined by significant recurrence rates and unpredictable hearing outcomes.

Rather than being the rarity it was once thought, CC is regularly reported in large series2-10 and, consequently, we have a more accurate understanding of its clinical presentation. The typical patient is a 5-year-old boy with a variable history of otitis media, in whom a white pearly mass behind an intact tympanic membrane is otoscopically observed by a pediatrician or an otolaryngologist. The mass often appears as a solid round white pearl in the anterior superior quadrant of the middle ear, just in front of the malleus manubrium. Hearing loss is rarely a problem. However, the lesion does not consistently remain in the anterior superior quadrant; it may variably occupy the remainder of the middle ear, with extension into the attic, antrum, and mastoid, and may result in ossicular destruction with conductive hearing loss.

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Understanding the spectrum of disease is the best guide for selection of the optimal surgical approach to eradicate the disease and restore hearing. The purpose of this report is to describe our experience with CC in a large number of children. Furthermore, analysis of the data may aid in defining a classification system of CC that will be a practical and reliable guide for predicting the success of a hierarchy of surgical interventions, the need for reexploration, the probability of recurrence, and the expectation of hearing restoration.
PATIENTS AND METHODS

Medical records of all pediatric patients with CC since January 1, 1989, at 4 children’s hospitals (The Cleveland Clinic Foundation, Albany Medical Center, Hospital Armand trousseau, and La Timone) have been independently and continuously maintained by 3 of us (P.J.K., E.N.G., and J-M.T.). From these records, all cases of CC were selected for inclusion in the study.

Congenital cholesteatoma was defined as a white, pearly lesion behind an intact tympanic membrane, which after removal was identified on pathological examination as a cholesteatoma. Cases in which there was loss of tympanic membrane integrity were excluded. All patients with lesions behind an intact drum were included in the study population, including those with a history of acute otitis media, otitis media with effusion, or resolved otorrhea associated with acute otitis media.

The 4 institutions contributed 3, 31, 62, and 23 cases, respectively, comprising 119 children with CC (age range, 2-14 years) whose medical records form the basis of this analysis. The records were of sufficient depth and compatibility that a unified database could be derived from them. The variables in the database included:

1. Demographics (age and sex).
2. History (acute otitis media, otitis media with effusion, and pressure equalizing tubes).
3. Examination (side, location [anterior superior quadrant, posterior superior quadrant, anterior inferior quadrant, posterior inferior quadrant, attic, and mastoid], ossicular involvement, and decibels of hearing at presentation).

RESULTS

One hundred nineteen patients with CC were treated. Of these, 78 (66%) were boys. Two children had bilateral CC; therefore, 121 ears were treated. The mean ± SD follow-up was 2.2 ± 2.3 years from initial diagnosis, at a median age of 1.2 years (range, 1 month to 11.4 years). The mean ± SD age at diagnosis was 5.6 ± 2.8 years. The mean ± SD age at diagnosis for acquired cholesteatoma, obtained from a separate analysis of 991 patients from the 4 institutions, was 9.7 ± 3.3 years. The age difference between children with congenital vs acquired cholesteatoma was statistically significant (t test, P < .001).

At presentation, 58 (49%) of 119 patients had a history of acute otitis media, serous otitis media, or both. Twenty-five percent had undergone myringotomy and pressure equalizing tube placement before treatment for CC. In most cases, the cholesteatoma was diagnosed at the time of surgical tube placement.

On initial physical examination, the left side was involved in 54% of cases and the right in 47%. Extent of disease was characterized at the time of initial tympanotomy. The involvement of cholesteatoma in 6 anatomical areas is summarized in Table 1.

Ossicle involvement was diagnosed based on erosion or loss of the ossicle, which was determined at the initial surgical procedure. The incus was eroded in 84 ears (69%). The stapes superstructure was missing or eroded in 69 ears (57%). The malleus, often surrounded by cholesteatoma, was eroded in 45 ears (37%).

Exploratory tympanotomy was the initial staging procedure in all patients. This alone was sufficient to eradicate the cholesteatoma in 43 ears (36%). Seventy-three ears (60%) required a canal wall up tympanomastoidectomy to clear the ear of disease at the initial surgery. A canal wall down procedure was required in 5 ears (4%). A second-look procedure was completed in 79 ears (65%). Of this number, 36 were exploratory tympanotomies, 34 were canal wall up tympanomastoidectomies, and 9 were canal wall down procedures. There were 10 third-look and 2 fourth-look procedures. Twenty-two reexplorations were being planned at the time of this report.

Preoperative hearing data were available for 111 of 121 ears. The mean ± SD hearing loss was 36.1 ± 18.3 dB. Follow-up hearing data were available on 105 ears, with a mean ± SD hearing loss of 26.3 ± 16.9 dB.

We defined recurrence as residual or recurrent disease. Thirty-eight recurrences were encountered in 33 ears by the second or third look.

Recurrence and initial hearing loss data are shown in Table 2. Patients are divided into those with CC limited to the middle ear space without spread to the posterior superior quadrant or the ossicular mass, those with disease extending to the posterior superior quadrant or attic, and those with mastoid involvement at the time of diagnosis.
The frequency of recurrence and the mean initial hearing loss increased as the extent of disease progressed from the middle ear to the mastoid (Table 2). Overall, these differences were statistically significant, but the pairwise comparisons did not distinguish between involvement of the posterior superior quadrant or attic from mastoid involvement with respect to frequency of recurrence.

A secondary question of interest was whether hypotympanic involvement had any association with recurrence or mean initial hearing loss among patients with posterior superior involvement. Recurrence rates were 43% in patients with inferior involvement and 37% in patients without inferior involvement ($\chi^2$ test, $P = .55$), and the mean ± SD initial hearing loss in the 2 groups was 42.0 ± 15.9 dB and 36.1 ± 17.2 dB, respectively ($t$ test, $P = .12$).

![Table 1. Involvement of Anatomical Areas by Cholesteatoma at Diagnosis](https://jamanetwork.com/)

<table>
<thead>
<tr>
<th>Region</th>
<th>No. (%) of Ears</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anterior superior</td>
<td>103 (85.1)</td>
</tr>
<tr>
<td>Anterior inferior</td>
<td>57 (47.1)</td>
</tr>
<tr>
<td>Posterior superior</td>
<td>97 (80.2)</td>
</tr>
<tr>
<td>Posterior inferior</td>
<td>65 (53.7)</td>
</tr>
<tr>
<td>Attic</td>
<td>78 (64.5)</td>
</tr>
<tr>
<td>Mastoid</td>
<td>32 (26.4)</td>
</tr>
</tbody>
</table>

**COMMENT**

The progression of growth of most CCs is from the anterior superior quadrant, initially into the posterior superior quadrant and the attic (the site of the ossicular mass) and finally into the mastoid. The sequence of spread involving 3 distinct anatomical sites suggests a natural hierarchy for the purpose of classification: Type 1 lesions are those that are confined to the middle ear but do not involve the ossicles, except for the malleus manubrium. Type 2 lesions involve the ossicular mass in the posterior superior quadrant and the attic. Type 3 lesions involve the mastoid. The validity of this classification is supported by our data, which show a recurrence rate of 0% when the CC involves only the middle ear without contact with the ossicles (except for the malleus manubrium), a 34% recurrence rate when it also involves the posterior superior quadrant or attic, and a 56% recurrence rate when it also involves the mastoid. There is a statistically significant difference ($P = .001$) in the progressively increasing recurrence rates at each of these anatomical sites, which parallel the growth of CC. This classification is further supported by the functional hearing losses of 12.5 dB when the lesion involves the anterior superior quadrant, 35.9 dB when it also involves the posterior superior quadrant or attic, and 47.7 dB when it involves the mastoid. There is no convincing evidence that hypotympanic involvement is related to recurrence or initial hearing loss once the posterior superior quadrant is involved.

In this report, recurrence refers to the regrowth of cholesteatoma, presumably from microscopic residual remnants of the previous surgery found at a subsequent exploration. Other terms for this type of tumor are recidivistic or residual cholesteatoma. We have not seen the type of recurrence described by Parisier and Weiss, in which a primary acquired cholesteatoma forms due to chronic eustachian tube dysfunction of the reconstructed tympanic membrane following surgery for CC.

When the CC is confined to the middle ear, without ossicular involvement (type 1), it is readily approachable by an extended tympanotomy, with elevation of the tympanic membrane off of the manubrium and umbo of the malleus. This access allows for the removal of the entire intact cholesteatoma pearl from the anterior mesotympanum, with a low probability of residual disease. The 0% recurrence rate suggests that type 1 lesions do not routinely require a second-look reexploration. There is generally no hearing loss with middle ear involvement unless the CC obstructs the eustachian tube, resulting in otitis media with effusion. Therefore, hearing restoration is a goal that can be achieved by removing the cholesteatoma by an extended tympanotomy.

The symmetrical spherical geometry of type 1 CC is broken as the superior and posterior vectors of growth push it up against the manubrium and neck of the malleus and the complex topography of its supporting ligaments. The lesions variably extend into the posterior quadrant underneath the malleus and may grow into the attic, probably because the origin of most CC is anterior to the manubrium of the malleus.

Type 2 CC, like type 1, can be approached via an extended tympanotomy, but removing a thin rim of scutum may be necessary to fully visualize the lesion. Teasing it out en bloc from under the malleus manubrium, and especially away from its neck and the cochleariform process, can be difficult because of obstruction of the view by the posterior extension of the lesion. Otolloscopy, with a 30°, 2.7-mm telescope, can often be helpful. However, to gain access to this site, it may be necessary to mobilize the malleus, which can be done via an atticotomy and separation of the incudomalleolar joint, which then allows the malleus to be rotated forward. Type 2 CC can result in osseolysis of the incudostapedial joint and may extend into the facial recess. For these lesions, a canal wall up tympanomastoidectomy with a facial recess opening is appropriate. The 34% recurrence rate reflects some of the technical difficulty in extracting the CC from these sites and warrants a second-look reexploration 9 to 12 months later.

Type 2 lesions can be associated with varying degrees of conductive hearing loss secondary to dampening of malleus manubrium movement by the CC underneath it, as well as by functional involvement of the incus and the stapes. With type 2 CC, the mean preoperative hearing loss was 35.9 dB and the postoperative hearing was 25.4 dB. This demonstrates the variability of hearing outcome with type 2 lesions.

Type 2 lesions involving the attic typically involve all of the ossicles and extend posteriorly into the facial recess and inferiorly into the sinus tympani. Control of the CC typically requires access to the anterior epitympanum, the facial recess, and the hypotympanum. This can best be accomplished via a canal wall up tympanomastoidectomy with an opening of the facial recess. If the ossicular chain is intact, an atticotomy is necessary to expose the head of the malleus. If the incudostapedial joint is eroded, the incus and the head of the malleus and attic are mobilized and a new incudal joint created.
Congenital cholesteatoma can be removed to get to the anterior attic via the mastoidectomy. Otoendoscopy is helpful for visualization and control of disease at these sites. The success of ossicular reconstruction for hearing restoration depends on the degree of ossiculolysis and the experience of the surgeon. It is generally more successful if the stapes is intact and an incus interposition is possible. When the stapes is absent, a sterilized and sculpted malleus or incus or an alloplastic total ossicular replacement prosthesis can be used for hearing restoration. The ossicular reconstruction can be done at the time of the primary surgery or at the second-look reexploration 9 to 12 months later. When the ossicular reconstruction is deferred, some authors advocate storing the ossicles removed during the primary surgery in alcohol and sending them home with the patient. However, we have found it useful to store the ossicles in the tip of the mastoid, from which they can be retrieved during the second-look procedure.

Type 3 lesions involving the mastoid also invariably occupy the entire middle ear cleft and have associated ossicular destruction and maximal conductive hearing loss. These are best approached via a tympanomastoidectomy, and all the points that were stated for type 2 lesions are applicable. The controversy of whether to take the posterior canal wall down or not deserves some comment. Rizer and Luxford argue against a canal wall down procedure, stating that a radical operation leaves a child requiring ear care for life. Grundfest et al state that CCs usually occur in children with well pneumatized mastoids, so a canal wall down procedure will result in an undesirably large mastoid cavity. Parisier and Weiss advocate a more flexible approach, applying the same principles for using a canal wall down operation to congenital and acquired cholesteatoma cases. Our experience is that the need for canal wall down procedures is rare, and this procedure should be considered only when there is destruction of the posterior canal wall, labyrinthine involvement, petrous apex extension, or concern about the reliability of follow-up. We agree that canal wall down procedures should be avoided, if possible. Nevertheless, when a canal wall down tympanomastoidectomy is indicated and performed with a concurrent wide meatoplasty, it typically results in a small, readily cared-for mastoid bowl. Although it is true that the mastoids of children with CC are well pneumatized, they are not as well pneumatized as the contralateral, non-diseased mastoid. However, more relevant to the dearth of mastoid bowl problems is the youth and consequent small mastoid size of children with CC.

The weakness of our classification system is that it imposes static categories on the dynamic process of CC growth, which occurs with a smooth transition between areas of involvement. Moreover, there are hierarchical inconsistencies between the extent of anatomical spread and the degree of functional impairment, most notably as the lesions expand posterosuperiorly and into the attic. Hence, in patients in whom there is inconsistency between the extent of disease and degree of hearing loss, surgical judgment becomes critical and conservative caution necessary. We acknowledge these limitations, but nevertheless our analysis has demonstrated that the proposed classification system is a reliable guide for surgical intervention, reexploration, recurrence, and hearing outcomes.

CONCLUSIONS

1. Congenital cholesteatoma usually develops in the anterior superior quadrant in young children, but does not consistently remain there and may variably occupy the middle ear and mastoid and result in ossicular destruction and conductive hearing loss.

2. The sequence of spread involving 3 distinct anatomical sites suggests a natural classification system.

3. Type 1 lesions involve the middle ear, without contact with the ossicles. Type 2 lesions involve the posterior superior quadrants and attic, the site of the ossicular chain. Type 3 lesions involve all of the previous sites and the mastoid.

4. As the type progresses from 1 to 3, the frequency of recurrence increases.

5. Type 1 lesions are controlled by extended exploratory tympanotomy and do not require a second-look reexploration.

6. Type 2 lesions are approached by an extended tympanotomy, but may necessitate atticotomy and canal wall up tympanomastoidectomy, with or without opening of the facial recess. They require a second-look reexploration. Ossicular reconstruction may be necessary.

7. Type 3 lesions are approached in the same manner as type 2 lesions, but occasionally necessitate a canal wall down tympanomastoidectomy.

8. When canal wall down tympanomastoidectomy is necessary, the small mastoids of young children with CC ameliorate mastoid bowl problems if an adequate meatoplasty has been performed.
Accepted for publication January 9, 2002.

This study was presented at the 15th Annual Meeting of the American Society of Pediatric Otolaryngology, Orlando, Fla, May 10, 2000.

We greatly appreciate the efforts of Scott J. Beam in the preparation of the manuscript.

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REFERENCES


Correction

In the article by Jang et al titled “Mucociliary Transport and Histologic Characteristics of the Mucosa of Deviated Nasal Septum” in the April 2002 issue of the ARCHIVES (2002;128:421-424), the legend for Figure 1 was incorrect. It should have read as follows:

**Figure 1.** In scanning electron micrographs of the septal mucosa from each nasal opening in a patient with nasal septal deviation, the concave side (A) shows sparsely populated cilia (original magnification ×1990), and the convex side (B), densely populated cilia (original magnification ×2180).

We regret the error.